Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting on Wilson Disease

SUBMITTED PATIENT COMMENTS & SURVEY RESPONSES

Meeting Date: January 29, 2026; Report Date: July 29, 2026

The Wilson Disease Association (WDA) hosted the Externally-Led Patient-Focused Drug Development (EL-PFDD) Meeting on Wilson Disease on January 29, 2026. An online comment submission portal was open before and four weeks after the EL-PFDD meeting to ensure that as many voices were heard as possible. Comments submitted through this portal are presented in this document, and selected comments are included in the Wilson Disease *Voice of the Patient* report available through the Wilson Disease EL-PFDD website.

Multiple comments submitted by the same patient were clustered together. Comments were gently edited but not rewritten. Those who submitted comments are identified by their first name, and a second initial if there was more than one with the same first name, as well as their current age, age of diagnosis and their Wilson disease manifestations if known. Some community members responded specifically to discussion questions italicized in the text. The names of specific healthcare providers and institutions were redacted to maintain confidentiality. Comments submitted in another language were reproduced as submitted and translated with Google Translate.

Prior to the EL-PFDD meeting, the WDA conducted a survey of the Wilson disease community. The survey results align very strongly with the EL-PFDD meeting outcomes and additional comments from that survey are highlighted in this document.

Submitted Patient Comments

Ali Ali, parent of a child living with WD
We lost our eldest daughter and we didn't know why and the same symptoms came back with my youngest daughter. We sold all our possessions and sent blood samples to Germany, which made it clear that my wife and I are carrying the same genetic mutation, my daughter has Wilson disease and my son is healthy. We are fighting a tough fight to get medicine and do tests and we are hoping that there will be a cure. You probably know the situation in Syria, save my family if you can.

Alice, parent of a 25-year-old living with WD, hepatic
Of all the health effects of Wilson disease, which 1-3 symptoms have the most significant impact on you or your loved one's life?

My son is 25 and the biggest challenges are socializing with other 20-somethings given his alcohol and diet restrictions, GI issues with medication and ongoing liver fibrosis issues.

Angela, parent of two children living with WD, neurological
I am an interested mother of two adult children with Wilson disease. Would be nice for someone who had neurological Wilson disease and is left with the life changing physical and left local brain problems to have the support they need. We have found that each symptom is treated separately. We have been lucky in the meds and follow up treatments but are left alone with the other life changing affects from the Wilson disease. I wish there was a central place for those who have Wilson disease in our community to see the whole picture and be able to have the support they need. Thank you for all you do and having this it is so appreciated

Arjun, 30-year-old diagnosed with WD at age 10, neurological and psychiatric
After discontinuation of the study drug, my tremors and dystonia returned while I continued treatment with trientine. What I miss most is the sense of relief I had during the study—my constant search for solutions to manage my tremors and Wilson disease had stopped because the treatment allowed me to feel normal and at ease.

Avonne, 57-year-old diagnosed with WD at 52, neurologic and psychiatric
More racial minorities should be included in drug research to address racial health care disparities.

Benny, parent of a child diagnosed with WD one year ago
Trying to figure out abdominal pain and weak knee symptoms for my son. Diagnosed one year ago on trientene.

Brad, parent of a five-year-old diagnosed in utero, asymptomatic
Our son, Calvin, is currently five years old and was diagnosed with Wilson disease in utero. We are very fortunate to have known about Calvin's condition since prior to birth thanks to genetic carrier screening followed by a chorionic villus sampling (CVS) for confirmation. Since the diagnosis, we have received amazing, thoughtful care from the team at [*hospital name redacted*].

Calvin is an energetic, intelligent, curious and thoughtful little boy. He loves riding his bike, exploring outdoors, reading books and spending time with his family. Fortunately, he has not yet exhibited any symptoms of Wilson disease.

Calvin takes zinc twice a day (started when he was 2 years old) and is on a low copper diet. There are many ways to define and abide by a low copper diet - in our family, we approach it as avoidance of chocolate, mushrooms, nuts, and shellfish. There are times that Calvin does not want to take his zinc or where the dietary restrictions feel limiting, but overall, his disease is currently very manageable.

What is most challenging is that we do not know how Calvin's disease will progress and present over the longer term. We are hopeful that Calvin will remain asymptomatic or will only have very minor symptoms over his life. We are fearful that our healthy, vibrant child could ever be impacted by symptoms, which could interfere with his personal relationships, mental health, physical wellness, and school / work. We hope that Calvin remains committed to the medication

and dietary restriction throughout his life to minimize risk of symptoms from Wilson disease and/or that new treatments will emerge over time.

Carol, 79-year-old living with WD, neurologic
Of all the symptoms health effects of Wilson disease, which 1-3 symptoms have the most significant impact on you or your loved one's life?

The symptoms that impact my life the most are slurred and low volume speech, and joint pain and muscle tightness. My speech issues forced me to change my selected profession from teaching to accounting and often make it very difficult to communicate. And my joint and muscle issues have greatly affected my mobility as I grow older.

How have your or your loved one's symptoms changed over time? How has the ability to cope with the symptoms changed over time?

As I get older, my residual neurological symptoms are limiting my mobility and activities more and more. My husband is a big help to me, but he is older than I am, and I worry that I might need more help than he can give, and my illness will be too much burden for him, but he won't tell me and would suffer or die because of it.
What factors would be important in deciding whether to participate in a new research trial, or try a new treatment?

It's difficult to find a research trial that would accept someone who is 79 years old, like I am. If one would accept me, then the most important factor would be how far I would have to travel to participate, and how often I would be required to make those trips.

Catherine, parent of a child living with WD, psychologic
We are the lucky ones. Our son's Wilson disease was accurately diagnosed before severe liver problems developed. The first visible symptoms were psychological and we didn't get to the WD diagnosis for 10 months as most mental health professionals are not familiar with the pyschological presentation of WD. We didn't give up and thanks the the Wilson Disease Association we found the information we needed to get an accurate diagnosis. So far, trientine has restored our son's brain health but it requires diligence to adhere to the dosing schedule and causes side effects. Our son is taking one day at a time, keeping a close eye on his diet and keeping tabs on the lab work to make sure trientine is still working okay and not depleting too much iron, leading to anemia. He doesn't complain but does suffer from nausea and other side effects, which make it hard for him to function to the best of his ability. He has had surgery twice for a stress fracture that would not heal despite the fact he is young and leads a healthy lifestyle. It's likely that his inability to properly metabolize copper impacted his bone health and ability to heal. Ideally, new treatments would help patients produce enough ceruloplasmin to bind with copper and support healthy minds and bodies in Wilson disease patients.

Cathy W., sister of a 65-year-old, diagnosed with WD at 15, neurologic and psychiatric
Comment 3: It is difficult to understand why patients can continue to have worsening symptoms, despite being successfully de-coppered and when ongoing labs show acceptable levels.

Cathy B., living with WD
Comment 1: Not all physicians understand what the results of lab work mean (copper excretion while on zinc vs other chelation therapy) I go to the same physician as my brother; and the doctor seems to have a different understanding of urine copper numbers and urine zinc numbers for my brother than for me. It would be helpful as a patient to have a guide to give to our doctor.

Comment 2: Another worry is that in our family's history the various medications needed to be changed or adjusted. We started out on penacilamine, then moved to zinc; and some siblings did not work well with zinc so on trientine. But it 'feels like' there is no permanent long-term usage of one medicine.

Christine, diagnosed with WD at 33, hepatic, psychiatric
Comment 1: I was recently diagnosed at 33 years old, mainly hepatic symptoms. I've had symptoms my whole life from fatigue, depression, anxiety. In my later 20's I had labile mood and unexplained elevated liver enzymes as well as infertility.

What scares me most is the health of my children. We see how hard it is to get a diagnosis and we see the detrimental consequences of not having a diagnosis soon enough. I've started having my two-year-olds labs taken and my 9-month-old I'll have tested at a year as well.

I'm scared about how I'll progress and what milestones of my kids lives that I'll be able to be present for. I grew up with a disabled mother unrelated to Wilson disease and I know how hard that is on a child, I hope for my kids that's not the childhood they will have.

Comment 2: I was found to have two pathogenic mutations during genetic testing for IVF. Since my labs were seemingly stable and I didn't meet the score criteria I was monitored for a few years and believed to be a carrier. After my final pregnancy and the hormone drop, my labs went into Wilson territory and I advocated for a liver biopsy and I got my diagnosis at 33.

I am fearful that my kids will be diagnosed despite their father's genetic testing being normal. I am fearful for my future and what involvement I will have in my children's lives depending on how my disease progresses.

The diet (lack of nutritional info in foods packaging), the windows to eat during a busy schedule for med adherence have been challenging to adapt to.

I am hopeful that more clinicians can advocate for Wilson disease screenings especially with those that have mental health disorders. I am hopeful that there can be a more definitive test that can diagnose patients, I am hopeful that genetic testing evolves to capture more pathogenic variants so patients can have a full and confident outlook on their health, I am hopeful that newborn testing gets implemented and I am hopeful there will be medications that help us to live more normal lives outside of diet restrictions and meal windows.

Cory W., meeting attendee
WTX-101 was a miracle drug. What can the FDA do to stimulate development of anti-copper medicines that can cross the blood brain barrier?

Dan G., living with WD
I recently switched insurance and was again denied coverage for Syprine [trientine hydrochloride] and the trientine alternatives. This has now happened three to four times since 2022 due to insurance changes tied to new jobs. It's incredibly frustrating and unacceptable to repeatedly lose access to medically necessary treatment because of insurance transitions. I am asking for support and advocacy to help resolve this issue as soon as possible.

Dhanya T., living with WD
Short of a complete cure, what specific things would you or your loved one look for in an ideal treatment for Wilson disease?

How to control anxiety. When I talk i talk very fast especially in tense situations.

Elizabeth, parent of a child living with WD, hepatic
Of all the symptoms health effects of Wilson disease, which 1-3 symptoms have the most significant impact on you or your loved one's life?

Extreme fatigue, abdominal pain/GI issues, and anxiety are the most impactful symptoms of WD. Working and sleeping are nearly all my daughter can manage. Her symptoms have progressively worsened over time. She currently hasn't the capacity for exercise, recreation, travel. An hour or so a week with friends is her only life beyond work. She worries she will not be able to work long term and be destitute. I worry about neurological issues arising, how her body will have deteriorated before a liver transplant, availability of a liver, rejection of transplant, and her general quality of life.

What are you currently doing to manage your or your loved one's Wilson disease symptoms?

Currently taking Trientine and zinc. As only 250mg/day of trientine can be tolerated (25% of recommended dose), WD continues to progress. Inability to tolerate full dose, very high cost (full dose would be crippling financially), inconvenient to take (away from food), and possibly associated GI issues are a few of the drawbacks to the meditations. Clearly tolerable and affordable would be awesome.

Erol, 65-year-old diagnosed with WD at 13, hepatic
Comment 1: Short of a complete cure, I would appreciate a one-a-day medication dosing regimen. If multiple doses are required throughout the day, then the ability to take the medication regardless of food and drink intake would be helpful as well. At the present I am on zinc salt and penicillamine. I have to take both meds away from food and away from each other, which makes consistent dosing difficult.

Comment 2: I want to highlight something important about emerging therapies. There are gene-based trials underway, and I volunteered for all of them. But I learned I have antibodies to the viral vector used in two approaches, and my specific ATP7B variant is not one of the common mutations targeted by the gene-editing approach. Many patients will face similar barriers, meaning we will still need effective, practical long-term medicines.

Eva, parent a 15-year-old recently diagnosed with WD, neurological
Comment 1: My 15-year-old son just got diagnosed with neurowilson. He is a US citizen, but we live in Hungary and there is little experience here unfortunately. Any info would be appreciated! Thank you!

Commnt 2: My 15 year old son with neurological symptoms living in Hungary is still struggling to receive the proper treatment despite of worsening symptoms.

Ginta, 33-year-old diagnosed with WD at 21, hepatic, neurologic and psychiatric
Watching everyone's journey today has shown me that no two experiences with Wilson disease are the same. Patients respond to treatments differently because every body is different. One of the most urgent unmet needs is improved insurance coverage for effective therapies along with expanded compassionate use access to clinical trial medications. I personally experienced a remarkable recovery while taking an investigational study drug and I often wonder how many lives could be meaningfully improved if more patients had access to these therapies.

Hayley, parent of a 14-year-old diagnosed with WD at four, hepatic
Of all the symptoms health effects of Wilson disease, which 1-3 symptoms have the most significant impact on you or your loved one's life?
Most significant symptom would be fatigue / low energy levels / weakness, nausea, headaches, stomach cramps.

How does Wilson disease affect you or your loved one on best and on worst days?
Best days: no symptoms (and elation when blood results are good). Worst days: symptoms as above and despair and worry when blood results have dipped

How have your or your loved one's symptoms changed over time? How has the ability to cope with the symptoms changed over time?
Symptoms have improved since diagnosis and start of treatment. No more chronic nosebleeds, better appetite, less nausea, better energy levels, huge improvement in liver function shown in blood work.

Are there specific activities that are important to you or your loved one that you/they cannot do at all or as fully because of Wilson disease?
Activities that require physical fitness, strength and coordination are a struggle. Having to be mindful of what can and can't be consumed with a compromised liver and avoiding high copper foods can be restrictive.

What do you fear the most as you or your loved one gets older? What worries you most about you or your loved one's condition?
I fear that my son will not adhere to the regimented medication regime and monitoring protocols as he becomes older and more independent which could cause him to regress. Medication adhereance is a daily burden.

What are you currently doing to manage your or your loved one's Wilson disease symptoms?
Zinc medication 3 x daily away from food, doctor check up every four months, blood work every three months, ultrasound every six months, urine collection every year.

How well do these treatments treat the most significant symptoms and health effects of you or

your loved one's Wilson disease?
Treatment has been successful so far

What are the most significant downsides to your or your loved one's current treatments and how do they affect daily life?
Downsides are having to take meds an hour before eating and having to stop eating for two hrs before the next dose, 3 x daily. It means continuous forward planning, watching the clock and timing all eating and activities around meds. It's a daily burden. Appropriate meds are also not easily available in South Africa. Galzin is unaffordable and Gluzin has to be imported. It's not a reliable or sustainable solution over the long term when my son will one day have to obtain it for himself.

Short of a complete cure, what specific things would you or your loved one look for in an ideal treatment for Wilson disease? What factors would be important in deciding whether to participate in a new research trial, or try a new treatment?
Ideal treatment would be one tablet / dose per day which can be taken with food.
Important factors would be safety and no risk of regression or worsening of symptoms and no significant side effects.

Helen, 55-year-old diagnosed with WD at 11
Comment 1: I take zinc sulfate with or after the food. No issues an no nausia. The doctor (he wrote a dissertation on WD, [*name redacted*], retired now) told us about taking with the food. Taking it from 1986 till now. Can someone maybe give a reaction now or in the mail later on?

Comment 2: Hi all, I am Helen, 55, diagnosed with wilson in 1982, age 11. I started penicillamine at age 15, I had severe side effects in the joints. Then i switched to 3 x per day zinc sullfate (200 mg). Still have that. I can do everything. my sister had WD too. She sadly died last August at age 52 due to cholangiocarcinoma and a tumor in the liver. There were no symtoms at all and WD was always stable.

Ian, 29-year-old diagnosed with WD at 12, hepatic and neurologic
Consistent access to affordable medication & healthcare for monitoring is crucial for long-term management & care for Wilson disease. There are very few, if any, viable alternatives to medication for chelation if a patient cannot afford or access them, as there are not many chelator medication options available (e.g. penicillamine, Trientine/Syprine). This is especially true if zinc salt therapies (e.g. zinc acetate) do not sufficiently prevent copper buildup during maintenance therapy for Wilson disease, which is lifelong. Patients need to have consistent access to medications & treatments in order to live healthy, successful lives with Wilson disease.

Irena, living with WD
Can we talk about the increase in price for Galzin? And how this medication went from $4.97 a pill to $49.21 a pill, for a SINGLE pill?

Because of the costs, my insurance has pushed for alternative options. I worry about the risk of just switching to another random medication that insurance is willing to pay for. It's ridiculous that we're willing to risk patients' lives!

Jason, living with WD for over 40 years
I've lived with Wilson disease for over 40 years now and I've been maintaining with medication's and a strict diet. Life is good however options for treatments remain to be very expensive with the current medication's being offered further clinical development in this rare disease area could benefit many needy people

JoRickard, meeting attendee
It seems as though Insurance companies are not up to date with any knowledge of what is Wilson disease. So I find myself explaining Wilson disease. Therefore, the call begins to go in the direction of educating the insurance company representation. This is a sad and stressful situation for the patient and their caregivers.

Judith, living with WD for more than 30 years, neurological
Just want to say that the TTM drug I received while participating in [*redacted clinical study*] blind study saved my life. My symptoms were neurological. I received it for 8 weeks. I am stable for almost 30 years on zinc and Copper Conscious Eating.

Karen, 63-years-old, diagnosed with WD at 18, neurologic and psychiatric
Comment 1: I am three years out from retirement. I am on Depen [penicillamine] to control my Wilson disease. My concern is coverage for this drug on Medicare. Not only is this drug expensive, most pharmacies are unwilling to get the drug for me. I have to get the drug through [*hospital name redacted*].

Comment 2: Concern: Drug coverage while on Medicare. Would a nursing home accept me because of high drug cost? A lot of pharmacies including Costco cannot even get Depen due to high cost.

Karin, living with WD more than 20 years
My worst symptoms is my speech issue it has not improved at all I have been diagnosed with WD more then 20 yrs ago

Kashish, caregiver for a 26-year-old living with WD, neurologic and psychiatric
My child has been diagnosed with neuropsychiatric Wilson disease at the office 10 years, since then she is on chelation of trientine, 4 capsules per day.

Since the start of cheletion her psychiatric symptoms worsen and was put on many mood stabilizer and antipsychotic drugs.

Now she is 26 years old but her psychiatric symptoms never improved. Now doctors say she has bipolar disorder, and given lithium and clozapine and electroconvulsive therapy also. She is in rehabilitation presently due to unmanageble symptoms at homes.

My question her psychiatric problem should have improved with copper chelation but in fact has worsened, is it due to side effect of trientine? Is there any other advance medicines available which reduces psychiatric symptoms?

Khanh, parent of three children living with WD, neurological
Comment 1: I have three children that are all diagnosed with Wilson disease. Two of them are symptom free and are doing well. Unfortunately, my middle son (16 years old) is severely affected with neurological symptoms, that we are all well versed in. He can NOT walk on his own, can NOT eat (uses GTUBE) and can NOT talk. He was previously an active 15 year old who excelled academically and was on the tennis and water polo team. We are trying to regain as much functionality as we can back.

We are hopeful that future treatments be made available that may help him (tetrathiomolybdate or its variants). My other two children are on low dose trientine and are doing well.

Comment 2: Please get the TTM approved for compassionate use in the USA !!!

Khushbu, parent of a child diagnosed with WD at 8
My son was diagnosed with the WD at the age of eight; now he is fine with the help of medication. I would love to know more about the latest research about the medicine.

Kimberly, meeting attendee
If you could discuss mood swings that WD patients experience and how the patient has dealt with them.

Kirk, living with WD
Comment 2: I've lost every job I ever had.

Laura M., 30-year-old diagnosed with WD at 7, neurological and psychiatric
What are the most significant symptoms of Wilson disease that you experience?

I was diagnosed with Wilson disease at 7 years old. Because I have lived with it for most of my life, many of my symptoms have felt "normal" to me, even when they were not.

The most significant symptoms I experience are neurological and psychiatric rather than visible physical symptoms. These include executive dysfunction, slowed cognitive processing, difficulty sustaining attention, chronic fatigue, emotional dysregulation, and periods of brain fog. While liver enzyme elevations have been present at times, the cognitive and psychiatric symptoms have had the greatest impact on my daily functioning.

How do these symptoms affect your daily life?

These symptoms have most significantly affected my academic and professional life. Executive dysfunction makes planning, organizing, and sustaining effort more difficult than it appears from the outside. Slowed processing speed and fatigue can make tasks take longer, even when I understand the material. Emotional dysregulation can also make stress harder to manage, particularly during periods of instability in treatment. Because these symptoms are not outwardly visible, they are often misunderstood, and I've tended to internalize them as personal failures and faults until I was educated about the wide range of symptoms patients can experience. Managing Wilson disease requires constant vigilance — medication timing, dietary monitoring, hydration, and laboratory follow-up. When combined with cognitive fatigue, this can feel like managing a full-time responsibility in addition to work or school.

Which symptoms are most burdensome or most difficult to manage?

For me, the most burdensome symptoms are cognitive fatigue, executive dysfunction, and emotional instability.

These symptoms affect my ability to maintain consistency in routines, which is particularly challenging because treatment itself requires consistency. The disease can make adherence more difficult at the same time that adherence is essential.

Because I have lived with Wilson disease since I was seven, it can be difficult to distinguish between my personality and disease related neurological effects.

Are there specific times when symptoms worsen?

Symptoms tend to worsen during periods of treatment interruption, stress, sleep disruption, or when laboratory monitoring is inconsistent. Even subtle changes in mental clarity can significantly impact academic performance and quality of life.

What would meaningful improvement look like to you?

Meaningful improvement would include sustained cognitive clarity, improved executive function, stable emotional regulation, and consistent mental stamina.

While normalization of my lab tests is important, improvements in focus, processing speed, and resilience under stress would have the greatest impact on my daily life and long term goals.

For me, Wilson disease has primarily been a neurological and psychiatric burden rather than a visibly physical one.

What are you currently doing to manage your Wilson disease?

I was diagnosed with Wilson disease at seven years old. I took D-penicillamine during my childhood, until around 15 years old when I switched to Syprine for relief of side effects like fatigue, weakness, and nausea. Until recently, I had been on and off medication for a couple of years due to insurance instability and high medication costs, before being aware of assistance programs and connecting with the WDA, but I am currently in the process of re-establishing care and starting Syprine again.

In addition to prescribed medication, I follow a low-copper diet, avoid alcohol, cook most meals at home, and bring my own water wherever I go. I also use a shower filter to reduce potential heavy metal exposure.

My cooking practices prioritize moisture-based, low-temperature methods such as simmering, stewing, and pressure cooking with adequate liquid. My goal is to reduce oxidative stress burden, support hepatic function, preserve protein integrity, and minimize advanced glycation end products associated with high-heat cooking. I also supplement with zinc, vitamin B6, and vitamin E, as I have been prescribed that trio since childhood.

How well do these treatments treat the most significant symptoms and health effects of Wilson disease?

When adherent to treatment and monitoring, my doctors report improved liver enzyme levels.

I have seen gradual improvement in cognitive clarity, emotional regulation, and overall stability while on treatment, particularly when combining medication with consistent dietary and lifestyle practices. I feel healthier overall when applying these strategies daily.

However, improvements have not been rapid. Because I have lived with Wilson disease since childhood, it is difficult to separate what is related to Wilson disease from my lifelong neurological baseline. Executive dysfunction, slowed processing, fatigue, and emotional dysregulation have been persistent challenges for me, particularly in my academic career. They remain the most impactful aspects of the disease in my daily life.

What are the most significant downsides to your current treatment and how do they affect daily life?

The most significant downsides include: refrigeration requirements for medication limit travel and flexibility, strict timing around meals requires constant planning, lifelong dietary restrictions and lifestyle modifications, and the high cognitive burden of organizing daily life around medication.

For people with executive dysfunction, the rigidity of treatment can compound stress. Managing Wilson disease requires sustained organization and vigilance, which can be challenging due to my neurological symptoms affect planning and focus.

Short of a complete cure, what would you look for in an ideal treatment?

Room-temperature stability and portability, flexible dosing not tightly constrained by food timing, effective copper control, measurable improvement in neurological and psychiatric symptoms, and reduced daily cognitive burden. For me, improvements in executive function, mental clarity, and emotional regulation would be just as meaningful as laboratory improvements.

What factors would be important in deciding whether to participate in a new research trial?

I would feel comfortable if there were strong neurological safety data, inclusion of cognitive and psychiatric endpoints, a regimen that reduces daily burden, clear communication of risks, and a realistic and affordable pathway to continued access if effective.

For me, the most significant impact of Wilson disease has been neurological and psychiatric rather than visible physical symptoms. Effective treatment would ideally address both copper control and meaningful quality of life outcomes.

Linlin, meeting attendee
The Eton version of Galzin shouldn't be only available via ANOVO. Eton should make Galzin available at main pharmacy chains, so we can get the medications at local pharmacies.

Liza, 45-year-old diagnosed with WD at 20
I was unable to attend the EL-PFDD meeting on the 29th, but would briefly like to share my experience as a Wilson disease patient. I was diagnosed at age 20, through genetic testing, after my sister became gravely ill with the same diagnosis. I have been living with WD for 25 years, and while I have avoided severe disease, my experience as a patient has not been without the significant struggle of medication compliance. For 20+ years, I battled the challenges of dosing Galzin three times daily, away from food. Much more, I struggled daily with GI side effects of nausea, heartburn and indigestion. This was particularly difficult throughout each of my three pregnancies, when I essentially discontinued taking Galzin altogether.

As an ALXN 1840 phase III clinical trial participant, I was beyond grateful for the time that I was able to avoid the daily battle and mental exhaustion of three times daily dosing, and subsequent GI side effects of Galzin. I was able to successfully take the study drug, once daily, and prior to eating, without any GI upset. My compliance was near 100% for the duration of the trial. My quality of life felt much improved, and it was much easier to navigate life as a mom of three boys, while maintaining part-time employment. It was utterly disappointing when the ALXN1840 trial was cancelled. While no longer taking Galzin, I still struggle daily with Cuvrior, twice a day dosing, and milder GI side effects. I am thankful for the WDA, the physicians, researchers, and all whom advocate for Wilson disease patients, and I am hopeful that ALXN1840 may be an approved treatment option in the near future.

Lonnie, living with WD for 39 years
Comment 1: I have been diagnosed with Wilson disease for almost 39 years. I am on Syprine for 39 years. I am very concerned about drug cost. I have struggled with confidence of being able to get the medication.

Lori, parent of a three-year-old diagnosed with WD at birth
Is there research being done on treatments in young children who are diagnosed through genetic testing at birth?

Currently my three-year-old who has Wilson disease cannot tolerate her zinc dose (she vomits on an empty stomach)

How do we know if medications are working? Before these children are potty trained and cannot do the 24H urine test - what is the best indicator that they are not accumulating copper? Is there any other treatments recommended for this age group?

Mackenzie, parent of a child living with WD
Short of a complete cure, what specific things would you or your loved one look for in an ideal treatment for Wilson disease?

Possibility of extended release zinc or alternative delivery that does not require restricting food intake around doses. I worry about this being a barrier as my daughter gets older and requires more doses throughout the day.

Maricela, living with WD for 44 years
I am from Costa Rica but now I live in Connecticut, my sister, a cousin and I were diagnosed in 1984 due to the death of another cousin, so far the only three of the family with the disease. I am happy to be [*name redacted*] patient and I take trianthene as a treatment. I am a mother of three boys of 29, 26 and 18 years old, all three healthy, thank God. I have always been punctual with the intake of my treatment and in these 44 years of illness I have not had major relapses, the most common thing in me is fatigue and sometimes my mood decays, when that happens I focus on enjoying the outdoors and nature and thanking everything I have been able to live and enjoy.

Melisande, living with WD

What are you currently doing to manage your or your loved one's Wilson disease symptoms?

My current treatment is combination trientene/zinc therapy. The amount of total doses in conjunction with the dosing guidelines makes meal time scheduling difficult. I have a routine and the slightest unexpected change is catastrophic.

Short of a complete cure, what specific things would you or your loved one look for in an ideal treatment for Wilson disease?

Ideal treatment would be one thing to take care of all the things (chelation and blocking), or drugs without so many interactions.

Michelle, living with WD
I had trouble finding the medicine in Lebanon. One pharmacy provided it at a VERY expensive price. Until God led us to WDA who have supported in ways we cannot thank enough for. Maybe it would be a good idea to have a WDA center somewhere in the Middle East for people here to access the medication and follow up on new treatments. Unfortunately, I had met a family whose children had got neurological disorders due to the absence of the medication. Thank you

Natalie, living with WD
Part of the reason why my family and I had to return to Europe after living and working in the United States for so many years was the fact that IN SPITE of the doctor having written a letter explaining that Galzin is NOT a "vitamin or supplement not covered by Part D", Medicare simply refused to add Galzin to my list of approved medications. That, and the fact that my Wilson disease- induced Multiple Sclerosis medication could also no longer be covered by a private entity (*redacted*) and I had to rely on the charity of strangers every year to see if enough funds had been accrued in order to open up the Medicare Copaxone fund... Medicare representatives telling me that Galzin was an OTC vitamin... And all of this because I was forced into Medicare after 24 months of being declared permanently disabled and in receipt of SSDI after losing my career as a K - 12 teacher. It's de-humanising. The impact it has on one's psyche and on one's immediate family is indescribable... Granted, my case was certainly a little more complicated because of the secondary MS (which was initially caused by the Trientine after Penicillamine gave me a severe case of S.L.E. that hospitalised me for two weeks in 2000). But "complicated" and "rare" don't make me something to be dismissed and discarded like trash in the eyes of a giant, faceless, entity. It's been a 30 year journey and I'm still here. I'll never let them defeat me. I say this on my own behalf and for every single other human being diagnosed with Wilson sisease and/or other rare and incurable illnesses.

...Losing my ten year teaching career and becoming permanently disabled still make me angry and depressed at times. I'm also an only child and my parents are in their late 70s. I have absolutely no support network beyond that of my mum and dad. The MS support groups don't have patients who also started off with Wilson disease. It's literally just me. I often feel completely isolated because having a rare illness is already quite lonely but having an even more rare combination of lifelong and life - altering diseases can make you feel completely and utterly alone in the world. Unseen. Chronically misunderstood. Bereft.

Nicki, living with WD
What are you currently doing to manage your or your loved one's Wilson disease symptoms?

I am currently in the clinical trial with Ultragenyx, Stage 1. The treatment appeared to be working and I went for 18 months without taking any WD meds. After that time, it turned out that the treatment was no longer working and my copper levels had increased. Now, I am on trientine and hoping to go back to the zinc that I had been taking for 30 years prior to the clinical trial. The hardest part of this, is planning meals or activities which include food around the medication schedule. I wonder why a treatment can't be developed which would increase the ceruloplasmin which seems to be the culprit in not being able to metabolize copper. It would be great to have a once a day treatment which even if it involved fasting, could be taken first thing in the morning.

Nicola, 38-year-old diagnosed with WD at 19, hepatic
I have lived with Wilson disease for over half my life (diagnosed following liver failure at age 19). Because I have been lucky to have great doctors, and because my body responded very well to trientine, I'm lucky to now live a relatively "normal" life -- liver function is stable, just need to keep taking (and affording) medicine and doing follow-up labwork and imaging. The exception to that stability, though, came when I wanted to have a baby. There is an amazing lack of information about safe dosages for both mom and the fetus/baby during conception, pregnancy, and breastfeeding. I'm lucky that I had a healthy son, but not without liver complications for myself (which have slowly re-stabilized in the years since his birth). My doctors at university research hospitals used their best judgment about managing my disease, but more targeted research in this area would be amazingly valuable.

Olivia, mid-30s, diagnosed with WD only six months ago, asymptomatic
I (mid 30s female) just got diagnosed with Wilson disease about six months ago through happenstance via regular prenatal genetic testing and follow up studies after it turned out I had two genes for Wilson disease. It turned out that my adult sister (late 20s) also has Wilson disease too. I'd like for research to work to better understand how different genes and gene combinations for Wilson disease impact patients' trajectory and outcome - how does someone with Wilson disease know what to expect depending on their unique genetic combination? I'm so surprised to learn I have this later in life and wonder what would have happened if I didn't have that general prenatal testing which identified the genes. It was very hard to find a specialist who knows what this is or how to help. I work in the healthcare field and really had to work to navigate getting into the right specialist to help. I can't imagine what this would have been like for someone with limited health literacy. I imagine it was also easier because I knew what I had genetically and wasn't trying to get diagnosed based on mysterious or unexplained symptoms. Again, more doctors need to know what this is and how to manage it - or at least what to look for and how to refer. We need more specialists so care is more accessible no matter where you live. I am about 12 hrs drive from my care team and only see them virtually. Sometimes I may notice a very fine tremor in my hands and occasionally have trouble swallowing but it's hard to know if that's Wilson disease or just normal daily variation. I'm only taking zinc right now thankfully but I feel like I've had more numbness and tingling in my hands and feet since starting it. It also hurts my stomach if I take it on an empty stomach, which makes it hard to take it as prescribed. That makes me less comfortable taking it and not knowing if the treatment is making things worse or not. The symptoms can be so severe and devastating that it is very worrying and I have definitely had more anxiety about my health since learning this, which in and of itself is hard. It gets easier as you go but you never know what will happen. I appreciate the chance to share this feedback and the FDA considering patient and family experience.

Patricia N., 70-year-old diagnosed with WD at 23, hepatic
I was diagnosed in 1979 at 23 years old. The hospital did not believe I would live long enough for a diagnosis. I was shutting down with liver disease. Then was diagnosed with the eye exam, blood test and 24 hour urine. Then the medication needed to be ordered as the pharmacy did not have Cuprimine in stock. I was finally almost there and by then vomiting bile and unable to eat much of anything while wearing maternity slacks due to the edema in my stomach. Slowly I improved and lowered dosage of medication. After a few years I was able to conceive my first child and then three more. The cost of my medication in 1979 was $26/month but by 2015 the cost soared to over $18,000/month and had I not found [*name redacted*] on my computer search I don't know where I would have turned. I am fortunate to do very well under his care. I am 70 years old today with my biggest complaint of making sure my medication is at least two hours after meals and one hour before eating!

Patricia V., 70-year-old diagnosed with WD at the age of 14, hepatic with minor neurologic
Although I participated in the panel discussion regarding future drug development and treatment of Wilson sisease, I did not share the complications/side effects of penicillamine.

In addition to the constant battle of a low white blood cell count, the physical toll on my body from the penicillamine has been tremendous. To date, I have had more than 22 surgeries under general anesthesia. The penicillamine destroyed my joints. At the age of 25, I began complaining about knee pain. I started taking over the counter NSAIDs eventually going onto prescription anti inflammatories. Knee pain does not support a career as an emergency nurse in a Trauma 1 level ER. In my 30's I was asking if there is a relationship between the joint pain and penicillamine. This was the early '90s. Then my feet started hurting and I walked like I was 80 years old- shuffling. It's very embarrassing to be stopped by everyone (coworkers, visitors, etc.), asking if I hurt myself. By this time I was working in the Texas Legislature where walking through the Capitol and hearing rooms is a fundamental requirement. Again, no answers. I did do acupuncture and herbs, which helped for awhile. In addition to the joint pain, my skin was sagging- hanging off my face, neck, upper arms- everywhere. And, I'm only in my 40s!

I had my first hip replacement at the age of 51. With each joint replacement, I have waited until it was almost impossible to perform daily tasks. In this instance with my right hip-it was when I had tremendous difficulty moving my foot from the accelerator to the brake.

I have had the following joint replacement surgeries: both ankles, both knees, both hips, both shoulders and interpositional arthroplasty in both elbows. I also developed severe scoliosis that was progressively worsening each year and had spinal fusion with rods and screws from T12-S2. Imagine not being able to bend at the waist, only at the hips? Life changing and life limiting.

Eventually, the osteoarthritis in my right foot got worse -it felt like a bone was coming through the sole of my foot. I had that foot reconstructed with a triple arthrodesis, unable to bear weight for 3 months.

The ankle surgeries had me non-weight bearing for 3 months each.

When my left hip was replaced, it was infected intraoperatively and my femur was cracked. This lead to the next two (2) years of having multiple surgeries, the hip removed, an antibiotic spacer put in place, multiple IV antibiotics for months via a port in my arm. Because I did not receive adequate surgical care in Austin and being told nothing is wrong despite the subsidence of the replacement, I eventually sought care in Houston where the majority of the surgeries, hospitalizations and follow-up took place. I share this experience because there are other "costs" or "impacts" from the penicillamine in addition to the "medical " side effects.

Five years after the infection, I was hospitalized again with sepsis due to that hip, with more surgeries and wily multiple IV antibiotics. In total that hip has been operated on 12 times. As a result, I have gluteus medius insufficiency- basically, I cannot walk up stairs using my left leg and cannot take more than 50 steps without my hip burning in pain.

At all times following every surgery, I returned to work within a few days- between 3-5 days after surgery. Depending on the surgery, I would take taxi cabs to and from work; use a wheelchair for mobility or a knee scooter and eventually a cane. I am sharing this not to be “heroic”, martyr, or victim; but I decided early after being diagnosed, that I would not let Wilson disease dictate my life. Unfortunately, I had to eventually retire from my job because I could no longer walk any distance.

In the next few weeks, I will have more knee surgery for a fractured patella. In June, I will have multiple joints replaced in my left hand. The osteoarthritis and rheumatoid arthritis has deformed my hand, caused chronic pain and caused a tendon rupture resulting in the inability to hold a pen, write, keyboard (I’m using one finger to write this- and the side of my finger because I have lost ROM in my wrist), dress, or cut my food. Forget about cooking.

I am thankful for surgical advances in artificial joints. And I am thankful for those surgeons are curious and work with me to continue my independence.

I respectfully request you consider the devastating effects of penicillamine and dedicate research towards a safer alternative. I also hope additional research in gene editing is supported; I believe this is the key to eventually eliminating this disease.

Rachel, caregiver of someone living with WD, hepatic
Comment 1: A member of my family was one of the first patients in the Wilson Therapeutics trial when Tetrathiomolybdate was known as WTX101. He was diagnosed later than many patients, and his symptoms were already severe and life-threatening with acute liver failure. Based on his symptoms, his medical team felt that the standard treatment protocol for Wilson disease would not be effective in time. WTX101 was his very first treatment, and it worked quickly to stabilize him when time was critical. Tetrathiomolybdate saved his life and was a successful treatment regimen for over five years.

As a caregiver, I know the terror of almost losing someone you love—and the hope you cling to when a viable treatment finally becomes available. My family was devastated when Tetrathiomolybdate was suddenly taken off the market and my family member’s current treatment is not as effective. I strongly support the approval of Tetrathiomolybdate so that other families can have the same chance at life that it gave mine.

Comment 2: If Tetrathiomolybdate became available, would you consider switching back from your current treatment?

Robert, living with WD
Comment 1: It's so interesting to hear other's experiences with this disease as it helps to understand and identify my own. Its true, but I never have thought too much about that my entire life I've had trouble with fatigue. I was constantly falling asleep in college, and at my job. At one point my French teacher threatened to throw me out because i couldn't stay awake through the class!

Comment 2: To echo others' comments, I would say that many decisions I made in my life were because of my Wilson disease. Where I could live. What kind of job I could have.

My freedom has always been limited by the meds, the constant lab work, and my dietary limits.

And wow, how strong are these people who can speak about this on the phone. I'm scared to cry publicly. Go Stacy!

Samantha, living with WD and parent of a nine-year-old living with WD, hepatic
My son is currently undergoing treatment for Wilson disease. He is 9 years old. Zinc alone was not sufficient and his condition deteriorated in 2023 with worsening liver function. He has been on a combination of trientine and zinc since then requiring 5 doses a day. Not only is this a massive burden for him, it is challenging for it to be managed at school and it is still not improving his liver function. I had a poor reaction to d-pen experiencing neurological symptoms that I had never experienced prior to the medication so we are very reluctant to change to it for him.

It was such a heavy moment finding out that the only other potential treatment was dropped from testing despite positive results and lifting the significant burden of medication dosing. Please consider the need for this to be reviewed for the wellbeing of many families and individuals. We are so scared about him starting to refuse meds or forget as he gets older and once a day would be so much more manageable

Samuel, friend of someone living with WD
My friend has been greatly impacted by Wilson disease, and so far many of the medications he takes have not been of much benefit. I remember sophomore year of college with him when it started to get worse that he had to drop out, which was eight years ago now. I learned about TMM tetrathymomibolate, and how it might be able to help his condition. I am writing to give support to him and in hopes that this medication can help my friend recover better.

Sanaa, 27-year-old diagnosed with WD in early adulthood, hepatic and psychiatric
I experience a lot of liver pain and general body pains.

Sandy, parent of two children living with WD for 37 years
The rising cost of the medications and insurance denial contributes to anxiety, continued insurance calls, doctor calls, nurse calls before approval to order. This medication keeps my children living life. 37 years being treated for both.

Sezin, 32-year-old diagnosed at 29, hepatic asymptomatic
I am 32 and was diagnosed three years ago without any symptoms. The only abnormal finding was consistently elevated liver enzyme levels in routine tests, possibly because the mutated genes involved in my disease are heterogeneous. I have been using trientine and zinc for the past three years, and my liver function and enzyme levels are now within the normal range.

This year, my doctor is gradually lowering the dosages. I have not experienced any side effects from the medications. I feel fortunate to have been diagnosed early.

I am an academic and an architect. I have follow-up controls every four months; although this can be stressful each time, I am currently well and plan to remain so in the future. I would also like to follow updates on medications and ongoing research related to the condition.

Sharon, parent of a son living with WD, hepatic
My son was diagnosed with Wilson disease in elementary school. We were lucky because he was being monitored for kidney disease (Alport syndrome) when elevated liver enzymes were discovered which prompted further investigation and ultimately genetic testing for WD. Challenges he has encountered include having to take medication during school, difficultly in keeping medication refrigerated during travel and recreational activities, finding doctors knowledgeable about WD, getting medication covered by insurance, understanding where my son is in his treatment since he has liver and kidney disease, side effects of medication (GERD, nausuea, stomach pain).

Stacy, 58-year-old diagnosed with WD at 24, neurologic and psychiatric
One driving force in my employment and career has been whether the employer offers health insurance that covers my medication. It's been the primary fact that determined whether I accept a job, stay with a less than satisfying employment situation, etc. The lack of affordable medication options has limited my life in this way, as I'm sure it's limited the lives of others. My freedom to choose a job should not have to be compromised based upon whether I can obtain my life saving medication.

Additionally, whether I could retire or not was determined by whether I could obtain and afford my medication. Thankfully, my employer offers retiree medical that includes prescription coverage. But others are not so fortunate, and the may have to continue working into their later years simply to have access to their medication.

Tania, living with WD
Is it normal to always be tired even when on lots of meds and I’m stable. Is it the disease or the trientine meds?

Troy, living with WD, neurolgic
I tried accessing the FDA call but was not accepted in, thank you for taking my feedback through the website. I have had great success with neurological Wilson disease treating with DMPS (2,3-dimercaptopropane-1-sulfonate) in combination with zinc. However, because the medication is not FDA approved in the USA, I can no longer purchase it and all the pharmacies are out. I take 500mg of DMPS per day, and 200mg of elemental zinc. DMPS is what helped me start walking on my own again, but I only have a few days left and am devastated. I hope DMPS is a medication the Wilson Disease Association will help us advocate to have approved in the USA, it is a first line oral treatment used alongside zinc in many other countries and there is a lot of published research showings its benefits and efficacy. DMPS with zinc (taken separately) was game changing for me, I am beyond upset that I cannot purchase it anymore when it is a great medication for Wilson disease. I am also desperate to get TTM but it is still not approved or available. Neurological patients especially need better, more effective treatments that do not cause serious injury, and oral DMPS should be an option. It was life changing for me when used

with zinc, but now I am going to run out of it. TTM also needs to be made available, we have a right to fight for our lives and a right to try to recover from crippling disability.

William, living with WD
My recent experience with losing Galzin insurance coverage took a lot of navigation to sort out. After numerous calls and emails, I finally qualified for the Patient Assistance Program. The insurance company was not able to provide any good advice until I was actually denied coverage and submitted the formulary exemption request. Thankfully, I had extra Galzin along with Gluzin to supplement my needs during the gap in medication availability. There has to be a way to better provide advice and allow patients to be proactive in dealing with these issues. I was notified by my insurance company in early December that I would be losing coverage for 2026. I was notified able to proceed with any alternative means until the coverage was actually lost and I was due for a refill. This is extremely dangerous and the timing needs to be improved.

Yasamin, meeting attendee
We believe that trientine should be affordable and easily accessible for patients who rely on it for long-term treatment.

Yunus, living with WD, neurologica (translated from Turkish)
Comment 1: I am a Wilson disease patient. [wilso hastasyıyım]

Comment 2: After taking Trientine, the active ingredient in this medication, I developed dystonia; my body is very tense. [Ben Trinentin etkin maddesi ni aldık tan sonra ben de distoni yaptı vücudum çok kasıyılıyor]

Comment 3: Are there any new treatments for Wilson disease? [Wilson hastalığı yeni çikan tedavileri var mı]

WDA Survey Responses (pre-meeting)

Ade, 45-year-old diagnosed with WD at 14, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I am relatively asymptomatic other than abnormal liver function

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

None.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

It's been tricky as we have to make sure trientine is not mixed with food, which means I can only eat at certain time of day. Procuring trientine has been a challenge due to supply and price.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Better effectiveness and less dependent with food.

Aida, 40-year-old diagnosed with WD at 13, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

It affects when I have to talk and when I have to walk. I am improving both but I still have difficulties.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Walking is difficult for me because I have to concentrate a lot and sometimes I fall. Talking it is also difficult but I try it and I improve. To do all things I have to concentrate and it causes fatigue to me.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The most challenging is the physioterapy and speech therapy because I have to put a lot of effort in it and it is expensive. But it is necessary in my daily life.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

The ideal is no neurological effects and no matter to take the pill with food.

Alice, parent of 25-year-old living with WD, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Fatigue, low energy, mild depression, anxiety.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Sports and work activities curtailed due to fatigue.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

GI issues due to meds upsetting stomach, have to time meds with eating.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Fewer side effects, take pills less frequently.

Andrew, 55-year-old diagnosed with WD at 49, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My Wilson disease was diagnosed mainly due to elevated liver enzymes but I have not experienced severe liver damage. My neurological symptoms and psychological issues, other than swallowing problems, have improved with treatment. I can speak mainly about having my personality change dramatically and then seeing it slowly return to normal over my six years of treatment as an adult. My journey after diagnosis will offer hope to those patients who are struggling. I can also speak to misdiagnosis, insurance denials and other challenges.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Early in my treatment I had trouble with sleep, weight loss, and irritability that affected my relationships and my ability to work productively. Early in my treatment I had trouble with sleep, weight loss, and irritability that affected my relationships and my ability to work productively.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I take trientine daily, follow a low copper diet and have copper and blood tests done every 6 months. Adjusting to this lifestyle was challenging since I had always eaten pretty much whatever I wanted, didn't take meds or have regular doctor visits. Adjusting my lifestyle to a trientine schedule took time and effort as well. Over time I've moved from being irritated and angry about these changes to becoming grateful for them and for the discipline required to adhere to the protocol. Making WD meds, diet, and testing a priority in my life has helped me become a healthier and better person. This process has taught me that the healing I've experienced far outweighs the cost of being inconvenienced by diet, testing or side effects.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Ideally all WD physical, psychological and neurological symptoms would go away with treatment. However most patients have issues that can't be cured so leaning to live with these is very important. It would be nice to take medicine once a day instead of twice or to have a shorter period of fasting. If there were no digestive side effects this would help as well. Less testing and dietary restrictions would be ideal if possible also.

Anonymous, 30-year-old diagnosed with WD at 10, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I was diagnosed with Wilson disease in 2005 at the age of 10, initially showing hepatic symptoms. Since then, I have been on trientine and zinc therapy. In 2017, at age 22, I began developing neurological and psychological symptoms. I experienced severe head pain—like someone was hammering a nail into the center of my head or crushing my bones. Daily life became extremely difficult. I had drooling, tremors, dystonia, hallucinations, and insomnia. My shirt would get soaked within minutes from drooling, and even simple tasks like eating or putting on a shirt became almost impossible because of the tremors. I lost my independence completely.

In 2020, I participated in a clinical trial for the study drug ALXN 1840. Over time, I began to recover, and by 2021, all my neurological symptoms had disappeared. My cognitive function improved, and the drooling, tremors, and dystonia were gone. Unfortunately, I was devastated to learn that the study was discontinued in 2023. About a year later, my neurological symptoms returned. The stress of their recurrence led to Functional Neurological Disorder (FND), which made my tremors much worse. Currently, I am back on trientine and zinc therapy and undergoing treatment for FND. It has been a long and exhausting journey with Wilson disease. I moved to the United States in 2013 just to access trientine, which wasn't available in India at the time. My family and I have endured immense financial, emotional, and physical challenges throughout this battle, but I have always fought for my life.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Writing, eating, speaking, working.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

It's a harder regime to follow since im on both trientine and zinc theraphy

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Just one pill in the morning.

Ashok, 37-year-old diagnosed with WD at 7, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Managing because there is no cure so far for movement disorder.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

It has drastically impacted quality of life, social impact and long term impact on future life.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Tremendous impact on quality of life cannot take any employment inspite of completing higher education and long term impact socially with little possibility to get married.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Can't say but combination of medicines are being used from time to time based upon continuius review and monitoring regularly.

Avonne, 57-year-old diagnosed with WD at 52, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Tremors make it difficult to walk, do fine-motor activities such as buttoning my clothes, writing, enjoying art, and safely cutting up food in meal preparation. Difficulty speaking has caused others to perceive me as mentally challenged and to feeling sorry for me and not taking my

intelligence and abilities seriously; this caused me to severely limit both personal and professional contacts, causing me to miss seeing family and friends during holidays and missing out on at least two professional nursing education conferences.

Feeling unbalanced on my feet has resulted in an average of at least one fall per year for the last five years, leading to more potential brain injury and increased medical cost to treat related injury. One fall led to expensive treatment for a serious medical condition called complex regional pain syndrome (CRPS) type 2, a condition recognized as more painful than even cancer and that which has been called the "suicide disease," because the unbearable pain has led sufferers to commit suicide.I live with daily pain from osseomuscular Wilson disease, and since this problem is not often recognized and treated, I am left alone to treat it myself. In desperation, because of this and the CRPS, I have traveled nearly 1,000 miles by car to buy a medical-grade redlight therapy machine on Facebook Marketplace that was on sale for $3,500 just so I could have adequate pain relief from the osseomuscular pain. Swallowing issues led to hoarse voice and choking on liquids and foods, resulting in an expensive visit to the ER, ENT doctor, and numerous specialists. Irregular heart rate, chest pain, dizziness, hypothyroidism all led to visits to the ER. These events led to high medical costs. I had to resort to using credit cards to pay off the debt. Cognitive impairment was mild enough five years ago so that I could still teach and looked forward to pursuing a doctoral degree in nursing education so that I could expand my career as a nurse educator. Now, worsened cognitive impariment means I can no longer teach as a nurse educator and can no longer continue to expand my nurse educator career through finishing my doctoral degree in nursing education leadership. Emotionally, all these issues have worsened my already-easily irritated personality due to Wilson disease. This increased irritability makes me often unpleasant to be around friends and families and forces me to socially and professionally isolate myself for the sake of my and other's wellbeing. Thus, I have paid a heavy price personally and professionally due to neurologic, psychiatric, and other bodily system effects from Wilson disease.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

I can no longer pay bills independently due to poor memory and inability to do math. I have severely limited my grocery shopping and social and professional activities to just local areas where I can drive no more than five miles, because I get lost easily even in a familiar area if I have to turn around in the opposite direction. Short term memory loss has forced me to be supervised in cooking, because otherwise I burn foods.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I live in a rural community, so the infrastructure is not as well-developed as urban centers. Therefore, when Hurricane Helene hit, I had no power for three days...Gasoline was limited so it was hard get gas to buy ice. My local grocery story was even without power. Due to high medical bills from Wilson disease, I could not afford to buy a power generator. I relied on coals and portable stoves, but that meant exra money I did not have. Life was hard. I ate cold meals and bathed in cold water and did not have clean laundry for a while. This caused stress that exacerbated my already-easily irritable personality due to Wilson disease. This increased irritabilty caused strained family relations. My trientine hydrochloride was without refrigeration

for all the three days without electricity. The pharmacy that supplied this medicine was one that my health insurance company mandated me to use. This pharmacy, which I hereafter will refer to as the mandated pharmarcy, did not accept returns, refunds, and replacement of my trientine hydrochloride, so I was stuck taking this trientine hydrochloride that had been unrefrigerated for three days.

The rural city I live in has frequent storms and frequent power outage, so I live with daily worry that as we gear up for another hurricane season, I will likely be taking ineffective trientine hydrochloride again. This worry exacerbates the already-irritable personality I am having now because of the impact of copper in my brain due to Wilson disease. This increased irritability causes strained family relations, which I feel bad about, because I do not mean to be so easily irritated with my family...Initially, my symptoms had improved after I started trientine hydrochloride, but my improvement eventually became inconsistent. The power outage may have played a role, in that it made my trientine hydrochloride ineffective. There was another factor that may have contributed to my inconsistent improvement, and that is, at one time, the mandated pharmacy switched the formulation of my trientine hydrochloride to an unrefrigerated trientine hydrochloride that was not the FDA-approved trientine tetrahydrochloride formulation manufactured exclusively by Orphalan, distributed exclusively by Panther Rare pharmacy under the brand name Cuvrior... When I started noticing inconsistent improvement in my symptoms, I attributed it to the phenomenon that may occur when it is not unusual for symptoms to get worse in the beginning of treatment as the medication causes a release of copper into the bloodstream. That was my initial thought. However, the severity of symptoms kept increasing to the point where I started forgetting to take my trientine hydrochloride on time. This kept happening, and because, as explained previously, since the mandated pharmacy would not let me get a refund, return, or exchange of the trinetine hydrochloride, I had no choice but to keep going in a circle like this. The stress of having no way out worsened my irritability, causing me to be easily irritated with my family, causing strained family dynamics.

For some unknown reason, in the middle of all this, the mandated pharmacy switched me back to the refrigerated trientine hydrochloride. I took this formulation of trientine hydrochloride for a while, but symptoms kept getting worse. I was forgetting to take the trientine hydrochloride. I was leaving it out on the counter for hours and sometimes days, because I was forgetful and did not even take the medication. At the point, I asked the mandated pharmacy to switch me back to the unrefrigerated trientine hydrochloride that it had given me once, thinking that it was the FDA-approved trientine that does not require refrigeration, when in fact, it was not...That was when I learned the truth from my physician that the mandated pharmacy had not been giving me Cuvrior. Instead, it had been giving me unrefrigerated trientine hydrochloride. I then got my physician to get prior authorization for Cuvrior. I was denied the first time but was approved in appeal. The lack of transparency from the mandated pharmacy and the stress of the appeal process for Cuvrior exacerbated my worries. The approval for Cuvrior is only for 1 year, after which I don’t know if I will be approved again.This causes daily stress!

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other “benefits” would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

The ideal treatment would be having nothing that I must administer to myself... My ideal treatment would be a perfectly normal *ATP7B* gene so that I would be a normal person; I would live a normal life. There would be only lab work every 6 months to 1 year to check for free copper to ensure it was not building up to toxic levels. There would be no symptoms unless I was having them and my level of free copper was at a toxic level.

Bernice, 67-year-old diagnosed with WD at 36, hepatic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Living with vegans it's harder to plan meals. The anxiety is also an issue.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Balance issues, fatigue, mental stress.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

When I was first put on creatine and penicillamine my body rejected the meds and I ended up with bacterial pertinences was hospitalized for five days. I've been on zinc treatment for the last 30 years. I'm now beginning to have issues with taking the zinc on an empty stomach as I have been getting the dry heaves before eating.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

I don't know enough about the side effects. As I've been very lucky with the symptoms I'm just starting to feel them now.

Brock, friend of Joe who recently passed away from WD at the age of 29, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My friend, Joe, was diagnosed later in life. At times he was making irrational decisions that were not in line with his character. We know now it was related to the heavy metal toxicity from copper. Eventually he had problems with speaking, balance and fine motor skills. Delays in medication access only exacerbated the condition and he eventually found himself unable to walk or move which led to immediate hospitalization. As of July 2025, Joe has been hospitalized for 10 straight months with little to no improvement. For the past few months, he has been completely unresponsive and doctors say prognosis is poor.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

The ability to participate in daily activities was severely hindered and he was unable to hold employment due to his WD symptoms. He often needed assistance just to walk from A to B. He could no longer drive within weeks of his diagnosis.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment

approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The medications helped reduce copper levels in his body. Beyond that, he remains unresponsive, most likely because copper toxicity damage had possibly reached irreversible stages. Doctors now believe he could have damage to his brainstem.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

The biggest issue I've seen is medication costs create delays in patients getting access, especially when they cannot work. Therefore, they have no health insurance to apply toward costs. Finding ways to create affordable access and expediting access could possibly have helped my friend before he reached life threatening stages.

Calvin, 24-year-old living with WD, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

The stress of the diagnosis paired with the tremors I used to have led me to lose several jobs.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Using precise tools and writing can be difficult.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I have been able to get Syprine for free because of my low income but I do not know if the program will decide not to renew my prescription each year.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

It would remove the Wilson disease like a tumor.

Carol, 79-year-old living with WD, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Affect: limited mobility, level of independence, and ability to communicate orally.

Cathleen, 65-year-old diagnosed with WD at 11, hepatic, asymptomatic

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

For me, monitoring is most important. Diet and medication compliance secondarily. We lost a sister (37 years ago) to non-compliance and brother who was first diagnosed with WD had a liver transplant eight years ago at age 59.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Most challenging is timing of medication (currently taking zinc). Cuprimine, we were likely too young to really understand timing of that; and I don't know much about my brother's daily routines post liver transplant.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Once daily medication, affordability.

Cathy, sister of 65-year-old diagnosed with WD at 15, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My sister can no longer think through options, make decisions, or make a plan. If you ask her "do you want to go see that movie with me" her response is usually "I don't know. I'll think about it". And then you do not hear back from her.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

She can no longer appreciate the concept of time. She has missed many doctor appointments because they get canceled because she arrives too late.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Penicillamine has permanently changed her skin; she has reddish, scaly lesions in places.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Meds that don't need to be taken before food or after food or with food. She also has type 1 diabetes so managing the food in relation to meds is complicated.

Ed, parent of two-year-old diagnosed with WD at six weeks, still asymptomatic
What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

I'm a caregiver of a 2 year old - no symptoms have presented yet.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

We provide our 2-year old daughter with elemental zinc (via suspension) 2x daily. Sometimes depending on her mood - this can be easy or can be difficult. As we are trying to do our best to slow down progress of the disease, we are hyper-vigilant on her eating habits and making sure that we remember to give her the zinc 2x daily. The zinc needs to be refrigerated - and as we have only started giving her zinc the past couple months - we haven't done much travel.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

I am not super knowledgeable about chelation therapies since our experience so far is with zinc. The holy grail, in my opinion, would be some version of gene therapy/editing where a patient goes in for a treatment that lasts for years/decades/lifetime. Obviously, there are efficacy & safety studies that need to be satisfied prior to this being a reality - and as we've seen, it is more difficult in practice than theory (re: Vivet). The zinc therapy itself has not been very difficult, except for the refrigerated aspect. Therefore, a zinc therapy that doesn't need to be cold would be ideal.

Emma, 20-year-old diagnosed with WD at 17, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Prior to my diagnosis, I was experiencing a vast assortment of symptoms: hair loss, slurred speech, drooling, and a complete loss of my handwriting. After I began treatment, my symptoms began to worsen at an exponential rate: I had lost complete control of my fine motor skills, lost all control of my facial muscles (to the point where I couldn't even close my mouth), I had lost the ability to talk, walk, eat and drink completely, relying on a customized sign language only my parents could understand and a feeding tube.

When I began to recover, I underwent intense physical, occupational and speech therapy to regain all of my basic functions. I've been told that people can't tell I have a neurological condition but now my symptoms are hidden: I deal with crippling anxiety, depression and fatigue. But other than that, I worked immensely hard to regain what I lost.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

The main things WD affects are my ability to study or complete assignments at the same rate as my peers. Because I was 100% bedbound for 2 years, my back lost its ability to support itself in

the upright position for extended periods of time. I now have to take periodic breaks in bed in between assignments. Another big thing is my diet, I have to be extremely careful that there is no copper in any of the food I eat or the drinks I drink. I have to go out of my way to ensure that restaurants have copper-friendly food and drinks which is an immense hassle.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I was originally on penacillimine, for the first week of my treatment, which was the main cause of my worsened symptoms. Thankfully, I was allergic and was later put on trientine and zinc. These treatment medications had to be taken on an extremely strict regimen (taken x amount of hours prior or after food, taken x amount of hours prior or after x medication.) Juggling this regimen with physical, occupational, and speech therapy, would've been extremely difficult without the support of my parents.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

In an ideal world, there would be chelators that mask the "poison" of the metal they are chelating. Chelators removed the copper through my bloodstream and effectively poisoned every limb my blood coursed through to the point of near paralysis. My experience with Wilson disease would have been far less traumatic if the treatment medications hadn't worsened my neurological symptoms to the point where I was a shell of a human being, fully conscious, fully awake, fully aware of my surroundings in a body that no longer worked.

Erol, 65-year-old diagnosed with WD at 13, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Other than first presentation of liver disease/failure, symptoms have little affect on day-to-day living.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

50 years on in treatment, I still have difficulty reducing my copper load. Therefore I am strict on diet control and have recently started on zinc + penicillamine. Taking these two drugs apart from one another abd away from food is challenging.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Tiresome always watching what one eats and difficulty working in meds between meals.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

One pill a day would be great. Gene editing/repair would be amazing.

Eugeni, 37-year-old diagnosed with WD at 11, hepatic and neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I have difficulties to walk and talk, it is very difficult to understand me talking and I fall a lot of times.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

For example I am very nervous if I have to talk and I walk very slow. I need a lot of concentration to do both.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).
Now I am liver transplanted, so I take medication for the transplant. The transplant improved a lot my life because before I was very very bad.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

No neurological symptoms and a medication who can improve the liver. And no secondary effects.

Farzana, 35-year-old recently diagnosed with WD, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Vomiting, low platelets, low hemoglobin, low WBC, blood vomiting.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Negative.

Ginta, 33-year-old diagnosed with WD at 21, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I've learned how to manage my symptoms the best I can, but I still deal with some lingering effects of Wilson disease every day—like tremors, migraines, chronic nausea, anxiety, and fatigue. These symptoms can make daily life unpredictable and draining at times, even with treatment. Over the years, I've had to adjust how I pace myself, plan my days, and advocate for my needs, especially when it comes to work and social life. It's a constant balance, but I've gotten better at listening to my body and giving myself grace when I need it.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Wilson disease symptoms make it hard to keep up with work and social activities, especially on days when fatigue, nausea, or migraines hit. There have been times I've had to cancel plans last minute or push through work while feeling completely drained. It can be frustrating not being able to show up the way I want to, but I've learned to adjust and give myself some flexibility when I need it.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).
The most challenging part of treatment is remembering to take my meds every day, especially when I'm busy or not feeling well. Cuvrior has been easier to manage since it doesn't need to be refrigerated, and I can carry it with me, which helps me stay more consistent. That convenience has definitely made a difference in sticking to my treatment routine.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

If I could design my ideal treatment for Wilson disease, it would be something I didn't have to take every day. I'd also want fewer side effects and less monitoring, just something that works quietly in the background so I could focus more on living my life and less on my condition.

Hayley, parent of a 14-year-old diagnosed with WD at age four, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Luckily, my son is now stable and experiencing a good quality of life. He is always generally though, quite low on energy. When he was first diagnosed, he was very fatigued, often vomited when he tried to eat, was very skinny and had terrible nosebleeds.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Biggest impact is having to live by the clock and always consider timing around food. It makes sleepovers at friends houses and things like school camps very tricky. Also making sure to avoid other medication that is potentially harmful to the liver and knowing that when he grows up, he won't be able to enjoy a glass of wine or a beer with friends or a wild night out as a student, due to his liver condition.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

We are thankful that our disciplined approach to taking meds correctly and regular monitoring has resulted in my son now being in a healthy, stable condition. He was taking penicillamine twice per day and zinc three times per day, all away from food, which was very challenging and only allowed for small windows of time to eat. After three-and-a-half years, we could slowly start to wean him off the penicillamine and now he is on zinc three times a day, which is much more manageable but to have to live by the clock and not just be able to eat whenever he feels like it is still a burden. Luckily he experienced very little side effects to the drugs but the zinc can make him feel nauseous or give him a sore tummy after he takes it first thing in the morning.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

One pill a day and can be taken with food!

Ian, 29-year-old diagnosed with WD at 12, hepatic and neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

The neurological symptoms have made speech more difficult, which I use daily for work. Fine motor changes also make handwriting much more challenging. Changes in liver function tests necessitated getting surgery and changing my medication treatment regimen.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Speaking, writing, and performing music were most affected. I talk with kids for my work regularly in schools, and have to repeat myself to make sure I'm being understood. I also have to handwrite reports, which is challenging due to fine motor impairments. I also had to stop playing music instruments recreationally because of the fine motor changes from WD.
How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

My chelation therapy medication (trientine) needs to be refrigerated. This makes traveling significantly more challenging, as I need to make sure I have a refrigerated storage method to bring it with me. Otherwise I'm constrained to stay at home. The chelation medication also needs to be timed around meals, forcing me to plan when I can eat around when I take the medication. That said, the trientine does appear to be improving my liver tests. I was on Galzin previously, but the treatment eventually became less effective and was restricted in how I could access it from my regular pharmacy.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Improve neurological symptoms, not require refrigeration, and be cost-affordable and straightforward to obtain without logistical hurdles (e.g. insurance, needing to use a location-specific pharmacy).

Janet, 77-year old diagnosed with WD at 15, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My symptoms occurred in the first few years after diagnosis. In the beginning, I had liver failure.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

No daily activities are affected now.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The most challenging aspect of my medications is having to take them 4 x day, one hour before I eat or two hours after I eat. For me, with an active lifestyle, this is difficult.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

My ideal treatment would be the ability to take the medication...regardless of timing of distance before/after meals; faster action would be wonderful.

Jared, 36-year-old diagnosed with WD at 19
How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Though proven to be mostly effective, my experience taking trientine/Syprine has been challenging at times. The biggest challenge is the fasting element before and after therapy. This is no problem in the evening but is most challenging in the morning and mid-day (which I no longer do after losing 10 lbs in the first year after diagnosis)

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

The ideal treatment would be a stabilized (less temperature dependent/non-refrigerated) pill(s) that was taken once a day and didn't require fasting.

Jean Marie, 62-year-old diagnosed with WD at 16, neurologic and psychiatric

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I have both neurological and psychiatric symptoms. My neurological symptoms affect me on a daily basis I have difficulty with speaking and swallowing. I can speak clearly, however, it does not come naturally. I have to think about every word I'm going to say (in my head) then remind myself to speak slowly and enunciate my words. It is mentally exhausting and embarrassing at times. My psychiatric symptoms have gotten worse with age. I have anxiety and depression. I'm on medication for both.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Having to speak clearly and my anxiety. I try to hide my anxiety when I'm out with other people. I have an arduous time especially speaking in a group. Another neurological symptom is I'm unable to write clearly.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

My challenge is trying to get three doses a day all on an empty stomach. I get nauseous with most doses and don't want to take if I'm out in public.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

I would ideally like to have gene therapy so I could eliminate my symptoms. If that's not possible yet, it would be nice to have a one time a day dose or even an infusion every three months or less.

Jessica, 39-year-old diagnosed with WD at 8, hepatic and neurologic

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Presented 29 years after diagnosis. Has taken over my life.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Keeping up with daily tasks at home and work.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The dyspeptic injections help with cervical dystonia. Taking chelator one hour prior of eating food or two hours after is bothersome.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Taking the chelating therapy regardless of meal time.

Karen, 63-year-old diagnosed with WD at 18, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I had to drop out of college because of Wilson disease symptoms. It took two years to decopper and at that point I never had the opportunity to go back to college.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Activities with a lot of physical involvement. I use computer mailing labels since my handwriting is so bad.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Cost of penicillamine and lack of pharmacies to even order drug! Sudden shortages of drug. I am scared to retire and am concerned about Medicare coverage of penicillamine during retirement.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

A normal priced drug that all pharmacies are wiling to order.

Kim Mi, parent of a 34-year-old diagnosed with WD at 20, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Woody was a regular college kid with a fully functioning body when he was diagnosed at age 20. He now has severe dystonia which have left him with spastic quadriplegia, he has been tube fed for 13 years and is unable to walk or talk.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

All of them. Woody requires total care. He is tube fed, unable to walk, talk or swallow. He is "all there" though!

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment

approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

We would like access to gene therapy at some point and would love a good chelator that doesn't cause neurological worsening. He is taking zinc currently. He would also love access to long term physical therapy which isn't realistically available once you are an adult.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Less side effects (neuro worsening in our case) and faster action. Earlier diagnosis.

Kim, parent of a 26-year-old diagnosed with WD at 20, neurologic with minor hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Over the past six years, my son has gone from being in college to the inability to walk, talk, or perform any day-to-day personal duties. This has significantly impacted our lives—both emotionally and practically. These limitations have required full-time caregiving by his father and I, constant medical attention, and major lifestyle adjustments. The presence of a stomach tube has further complicated daily routines, requiring specialized feeding and increasing vulnerability to infections. Together, these challenges have altered our family dynamics, introduced financial and emotional strain, and required deep resilience and adaptation.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

My son is basically bed-ridden. Every activity is impacted by this disease. We were at one time able to bathe him daily. Then it went to every other day to sponge bathing him because he cannot sit in a chair within the shower without sliding out of it.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Physical therapy has been a vital part of caring for him. I provide him with some therapy and we pay for a professional therapist to come 2x a week. We have seen my son grow stronger and more flexible.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

I would love to see intensive physical therapy for an extended period of time. I believe all WD patients would benefit.

Lana, 46-year-old diagnosed with WD at 22, hepatic with minor neurologic

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My symptoms have improved dramatically since diagnosis. I was initially on the liver transplant list and lost the ability to read, write and slurred speech. A clinical trial saved my life. I have been in maintenance since that time, primarily on galzin. But have participated in other clinical trials.I do suffer from migraines which can be debilitating. I did have a reaction last year where my liver enzymes were severely elevated and I had to go on a high dose steroids for several months that caused a huge weight gain.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Daily activities are not a challenge for me currently. Years ago, all aspects of daily living were impacted.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Finding time to get all my doses of galzin can be difficult with travel, my job as an attorney when I am litigating and when I am feeling nauseous. I actually recently had an argument with another attorney at a deposition because I needed a three minute break as a result of taking my medication.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Best treatment would be a cure:-). But otherwise once a day, without worrying about nausea & stomach aches.

Laura D., parent of eight- and six-year-olds, diagnosed at four and two, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My 8 year old son was diagnosed at 4 years old and by then already had Stage II fibrosis. He never experienced outward symptoms. His liver enzymes took almost three years to normalize.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

WD has impacted him most in terms of diet and medication. Telling my young child he could not eat chocolate cake at birthday parties was very difficult. Hoping that he wouldn't eat something inappropriate at school worried me. Spacing out meals and medication doses is very difficult in a young child.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment

approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

My son had a skin reaction to penicillamine and switched to trientine. For a time he was on two doses of zinc a day along with two doses of trientine; this was very difficult to work into a young child's daily life. He is currently on only zinc but it sometimes makes him vomit so we are constantly changing his med schedule so that he can take his meds without getting sick while keeping copper levels in check.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

No refrigeration, fewer side effects, once a day dosing, dosing along with food to avoid stomach upset, my son was technically overdosing when he was on trientine because they do not make small enough doses for children.

Laura M., 30-year-old diagnosed with WD at 7, psychiatric
If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Probably gene therapy, as it would be nice to not have to remember to take medication, fast, or even have to avoid certain food groups that are culturally important for me.

Lee, parent of a nine-year-old diagnosed with WD at age four, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Because Wyatt is so young our struggles have been with medication management and diet control more so than symptoms.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

He can do most things, there just needs to be education of those around us for what he can eat and when he can take his meds. Halloween, Christmas, and birthday parties are hard.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

My son takes penicillamine - the most difficult time we had was when we switched insurance companies and they wanted us to change medications from what had been working for years prior.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

It would be nice if there was a medication he could take once a week or something of the sort, as the constant awareness of when he eats and takes his medication is exhausting. I would also love a way to check his copper more frequently, at home (like diabetes patients can do with insulin).

Linda, 76-year-old living with WD, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I am fatigued all the time.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

I can't do more than one big thing a day.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Penicillimine has kept my symptoms under control.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

One injection a month.

Mandy, 29-year-old diagnosed with WD at age six, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Was diagnosed early, mostly my experience is living with chronic disease, taking medication, attending doctor's appointments and what that was like from a young age/not understanding everything

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Taking medication at work/while in the operating room was very difficult when the medication was refrigerated, is a bit better now that non-refrigerated options are available. Still can be challenging to take medication when busy but easier now. Also attending doctor's appointments has been hard during college/medical school/medical training.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Most challenging to take meds in an environment that doesn't always allow drinking water etc. Speaking out has been helpful and advocating for myself.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Gene therapy will be amazing one day! Or being able to take the medicine with food, stronger medicine that allows us to eat "off limits" foods without guilt.

Maria, 41-year-old diagnosed with WD at 20, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Well they worry about me, specially since my liver has gotten worse in the last 4-5 years, they have taken me to ER many times because of the cirrosis....

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Exercise, social interaction, depression, no motivation at all because of fatigue.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Trientine is a well tolerated treatment for me and the most challenging aspet is to keep trientine cold while traveling. When i was under penicillamine, it damaged my body, specially neurologically. Another challenging aspect is the fastening thing specially when I'm out.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Fewer side effects, not having to refrigerate the med.

Mary M., 36-year-old diagnosed with WD at age nine, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

When I was little I felt physically ill and went into liver failure - once on penicillamine and therapeutic I lived relatively normally. Switched to galzine. About 20 years later while pregnant, had significant neuro symptoms - tremors, loss of balance, slurred speech. Had to go back on chelator. My sister also lives with Wilson disease and had mostly neurological and psychiatric symptoms.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

When I was affected neurologically I was working as a nurse in the NICU. Noticed it while putting IVs in babies initially. It progressed until I was off balance and slurred speech one day - thought I was having a stroke as my recent blood test had normal liver function. I ended up leaving the NICU and moved to outpatient nursing where I didn't have to insert IVs because I didn't want to not be able to perform my best. My career path changed because of my tremor I had even though it has since resolved.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I notice very minimal symptoms on true time. It is hard to take on an empty stomach. When I had been on Galzin I would often throw up

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Less dosing and able to eat chocolate!

Mary P., 49-year-old diagnosed with WD at 39, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

When I had acute symptoms (pre diagnosis and during treatment), I was completely disabled; unable to care for my daily needs. No one other than my immediate family could understand me. I was on zinc therapy for two months before entering a drug trial for tetrathiomolybdate, and extensive physical, occupational, and speech therapies, I was able to recover. Because the drug study ended abruptly, I am currently on standard of care taking trientine because I cannot tolerate zinc. Since taking trientine, my blood and urine test results have been unstable and some tremors have returned. I also suffer from generalized pain and fatigue. I can no longer work full time as a trial attorney because of the long hours and stress.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

When I had acute symptoms, I could not do anything for myself. I tried constant care. Currently, I am greatly limited in my daily activities because of fatigue.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

When I was on the study drug, my life changed drastically because I was able to function again. It was easy to take because regardless of dose, only one pill was necessary. When the drug study ended, I started on OTC zinc therapy because my insurance would not cover Galzin (and it was cost prohibitive to pay cash), I struggled greatly because of the rigidity of taking 3 doses away from food and the side effects. After trying for 6 months feeling absolutely miserable from constant nausea and need to vomit, I reluctantly started trientine. I worried about it's side effects on a neurological WD patient. Since taking trientine, the first time in my journey, my liver

enzymes were abnormal and I have not been able to stabilize my urine copper levels. The rigidity of taking multiple doses a day is frustrating and impacts my daily schedule. My pain levels are higher on trientine and some of my tremors have returned.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

This is a hard question to answer. As a fantasy, I would design a single dose medication that would appreciate all symptoms immediately and allow WD patients to live a normal life without dietary changes. Realistically, if the therapy didn't require immune suppression and would last a lifetime and actually alleviate WD symptoms, that would be ideal. Currently, if I could take one dose per month, and the drug would keep symptoms away, and allow me to eat anything (I'm a real foodie but highly compliant patient so I do not "cheat"), would be ideal.

Nicole, 41-year-old diagnosed with WD at 10, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I try not to let it affect me or my way of living.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Slurred speech and unsteady gait.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Galzin impacts my life positively. If it didn't then I wouldn't take it.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Yearly Injection.

Nicola, 38-year-old diagnosed with WD at 19, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

At this point in my life, I am stable and my liver is healthy, so my day-to-day life is more about maintenance (taking medicine, regular blood/urine/imaging tests, watching diet). I spent many years with very acute liver-related symptoms, which was very scary as a young adult. I also went through the process of pregnancy with Wilson disease -- so little information about safe amounts of medication, liver-related complications that resulted in needing an early induction. But amazingly have a healthy five-year-old now.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Currently none. There was a point in my life when I couldn't walk more than a block at a time or easily go up the stairs to get to my room.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The biggest challenge is timing of medication. At one point, I was on four doses a day, which meant I had to very carefully watch when I took them and limited when I could eat to just a few windows (since you can't eat for a while before or after). It's also challenging needing to keep the medication refrigerated (but not frozen) -- makes travel more complicated. That said, so grateful there's a medication I haven't had ANY side effects from that's let my liver heal.

Nora, parent of 14- and 10-year-olds diagnosed with WD at 18 months, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My oldest missed two years of school, returned this year and has made amazing progress on chelation. She did not present typically at all. She started with tremors, balance issues and a multitude of other symptoms dismissed as a functional disorder.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

All of them in the beginning, she couldn't stand in the shower, I had to bath her. Her schoolwork at home needed to be typed because she couldn't write well.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The girls have done every test, dietary modification, set timers for medication because the symptoms were so bad. Any medication to treat a symptom she tried, she did her research and is not afraid to advocate for Wilson disease patients. She never wants anyone else to go through what she did and nor do I. My 14-year-old has done several projects for school about her experience with the disease.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

The biggest impact would likely be not having to space out the medications between eating and other medications. They do great with that but hard with teens and a preteen.

Om, brother of 37-year-old diagnosed with WD in 2012, neurologic

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My brother is living with Wilson disease and he was diagnosed of it in 2012 December. Since then his life has never been normal, as his symptoms worsened day by day, he has been suffering with severe neurological issues which includes being unable to walk, unable to talk properly, severe skin issues, issues in swallowing food and also forgetting things as we remember in past, his memory was very sharp and great.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

I beleive every activity from getting up and going to washroom to reaching the dinning table and having food properly to speaking with his twisted tongue.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

At least he should be able to walk so that he doesn't feel he has to be dependent on anyone or a walking stick.

Patricia, 70-year-old diagnosed with WD at 14, hepatic with minor neurologic

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Prior to diagnosis at the age of 14, I was jaundiced, ascites, tremors, visual hallucinations. Following diagnosis, most symptoms resolved, however the treatment with penicillamine caused an autoimmune reaction including neutropenia, and multiple joint degeneration, chronic pain and fatigue. Mobility is extremely limited along with the ability to perform ADLs. Severe joint and bone degeneration in my hips, knees and feet, forced me to retire from my job.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Ability to prepare meals, dress, wash and toilet. Travel is impossible without a companion.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

I am excited about the gene therapy. To be saved from a lifetime of side effects from pharmaceutical treatments and complications of WD, including kidney and liver disease would be incredible.

Raewadee, 49-year-old living with WD

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I grew up with my brother who had neurological issues which were quite severe. I was diagnosed much later during difficulty getting pregnant.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Before treatment, had many ailments that I was too young to have, or not explainable.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Difficulty getting prescription filled.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Gene Therapy?

Raisa, 31-year-old diagnosed with WD at 17, hepatic, neurologic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

It was challenging to use utensils at family functions and kaiser volunteer appreciation luncheon. I lost all my medications during Hurricane Irma in Florida: I packed it away with ice when we evacuated to the nearest cat 5 secure hospital, but by the time I got there, it was soaked and unusable. I have also missed midterms/exams in college due to insurance issues where the pharmacy would not release my medications to me due to its egregious out of pocket cost. Both experiences exasperated generalized anxiety and contributed to trauma related symptoms.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Social activities that involve food, pursuance and completion of higher education including graduate/professional school.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Complex medication regimen, additional planning required for all activities.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Palliative/supportive care practices as soon as possible and integrating mental health care alongside the medications or liver transplant

Rhonda, 63-year-old diagnosed with WD at 21, hepatic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

They don't.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

None.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Good.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Curative.

Sanaa, 27-year-old diagnosed with WD in early adulthood, hepatic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I live in a very stigmatised state of life due to the debilitating psychiatric issues. I have been bullied for these psychiatric issues all my life. I only started to make healthier decisions after I was diagnosed in January 2021. I decided to stop anonymous chatting and do better in academics by returning to university.
What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Communication. I find it difficult to stand up for myself, to voice my opinions and I constantly doubt myself and get easily influenced in decisions that I should be making myself. For example, I am studying chemistry with the open university UK and my modules for next year are too many but I was pressured to take them because I am getting older and need to finish soon a lot of this is culture I come from an indus culture so Pakistani culture. Indian culture too.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

The constant feeling of others treating me poorly or not respecting me because they think I lack intelligence but I just love chemistry and I love chemical engineering too and want to excel. I am somehow very unmotivated most of the time due to studying online this way as I don't have friends to study with me but I manage to look on the bright side and think that I am developing independent learning skills. I take three zinc gluconate tablets a day and two penicilamine. I feel alright these days, I've been chelating for four years now. My tremors have reduced.

I hallucinate on a daily basis and it's usually a mix of fictional things I've watched and the reality is often very distorted. I feel like I'm in space and I can't understand how to protect myself when I go blind suddenly or when I don't perceive a sensation such as if someone touched me or grasped my shoulder to talk to me.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

I think my ideal treatment is a cure. I wouldn't wish for anyone to have this hideous illness. there is so much self hatred I experience because I can't let go of the many things I go through and the depression I feel when I can't eat normally such as avocados and chocolate. I think the medicine should focus on food such as us Wilson disease patients being able to eat as much of anything we like no matter the copper level. A drug that doesn't let food copper concentration affect well being would be the next best thing before the cure itself.

Sergi, 37-year-old diagnosed with WD at 11, hepatic and neurologic

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

I have difficulties to talk and walk.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

The most impacted is to socialize, it is very difficult for me to talk with people.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

Now I am liver transplanted, so I only take medication for the transplant. The transplant saved my life because my liver was very bad before.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

No neurological effects and improve the liver functionality.

Stacy, 58-year-old diagnosed with WD at 24, neurologic and psychiatric

Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

When I was first diagnosed, I had hand-flapping (like I was trying to work a hand puppet's mouth), tremors, balance issues, slurred speech, drooling and my handwriting became very small and illegible. I was no longer able to work (clerical/administrative work) and was let go from my job. It took about a year and a half of chelation for my symptoms to subside enough that I could return to work. My neurologist said that I could only work in a part time, stress free job - but unfortunately a part time job doesn't pay the bills nor provides health insurance, so I went back to work full time. Prior to my retirement two years ago (not related to WD or health reasons) I was working 60+ hours a week in a highly stressful environment. No one would've been able to tell I have WD.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Only activities that require a high level of balance are impacted. So I stay off ledges, high curbs, etc.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I have taken penicillamine since I was diagnosed in the early 1990's (I believe that was the only drug available at that time, or at least the only drug available to me through my health insurance.) Thankfully I responded well to the drug. My challenges over the years have been mainly with getting timely refills of my prescription. Although it is a maintenance drug, my insurance will only refill a 30 day supply at a time, so I need to make sure I stay on top of requesting my refills.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Maybe more attention to the behavioral aspects of the disease? I was prescribed penicillamine, and was basically told that it would take care of all of my symptoms once the copper was chelated. Which was true up to a point, but some support/guidance as I made my way back from the behavioral issues to "normal" would have been helpful.

Stuart, 27-year-old diagnosed with WD at 24, hepatic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

Fatigue has always been an issue. I had a really hard time getting up for school in the morning as a kid. I also had psychiatric symptoms but I'm not sure how much of that is Wilson disease related.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Getting up for school was the biggest thing, but I just have less energy to do stuff in general.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment

approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

I'm taking a maintenance dose of trientine. Honestly it's pretty easy, especially since I don't have to take it every day anymore.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

Ideally a cure but if it's just a treatment in pill form is preferable.

Teri, 36-year-old diagnosed with WD at 24 hepatic and psychiatric
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

My anxiety and depression makes it hard to get out of the house. Mood swings are crazy; I can go from happy to upset really fast. I'm in pain the majority of the time.

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Hard to leave the house, can't work more than five hours without pain or feeling exhausted.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

They've helped relieve some of my symptoms but also makes my stomach hurt really bad.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

No symptoms or less pain, wanting to feel normal. Once a day meds would be great.

Tina, parent of a 20-year-old diagnosed with WD at 17, neurologic
Briefly describe how these symptoms affect you or your loved one's day-to-day life, including how these symptoms have changed over time. Please share examples of when you or your loved one experienced these symptoms and the impact it had.

For example, this morning, my daughter was on her way to college, and she was extremely tired and fatigued—something most WD patients struggle with daily. She had to pull over and rest in a nearby parking lot until her body recovered enough to continue. Just last month, she nearly fainted in the shower. She also struggles with depression on a daily basis. It's incredibly difficult for anyone to navigate life when their body is constantly in pain and exhausted. As caregivers, we have to stay alert and be ready to provide both physical and emotional support. It's a constant responsibility, but one we carry out with love and determination."

What daily activities are most impacted by WD symptoms? Please share examples of how WD symptoms made these activities difficult or impossible.

Every chore, daily task, school or work responsibility, and even self-care has to be planned in advance. For WD patients, there's no way to predict how much their body will be able to handle the next day. If they don't get adequate rest, they often pay the price for days afterward. My daughter has learned how to prioritize her tasks carefully. On some days, that means letting go of certain activities in order to conserve energy. The most important thing is ensuring she isn't too exhausted to care for herself—especially making sure she eats her meals.

How would you describe your day-to-day experience on current treatment(s) for WD (e.g., what seems to help, what aspects are most challenging)? Provide examples of specific treatment approaches and your experiences with them, including how they impacted your quality of life (positively or negatively).

When my daughter was undergoing treatment, managing her medication and meal schedule was extremely challenging. Every medication had to be taken either hours before or after meals, which made planning incredibly complex. At that time, she couldn't swallow anything and had to rely on feeding tubes. Her earliest medication started at 6 a.m. and the schedule didn't end until 10 p.m., with three tube feedings in between. It was exhausting—not just for her, but for us as caregivers trying to coordinate everything. There truly needs to be a better option—perhaps a trial medication that only needs to be taken once a day. That would be life-changing, even life-saving, for anyone battling Wilson disease. Imagine being so sick you can't even lift a finger, yet you're required to go through a long list of physically and mentally draining steps—just to take your medication and eat. It's an overwhelming burden on someone already fighting so hard just to function.

If you could design your ideal treatment for WD, what would it look like? (e.g., what symptoms would it improve or what other "benefits" would you hope to experience, delivery mode, dosing, fewer side effects, less monitoring, faster action, other)

What we really need is a drug that is easier for patients to use—especially a chelation therapy that causes fewer neurological side effects. Many of the current treatments are not only complicated to manage but can also worsen symptoms in patients with neurological involvement. A more tolerable and simplified medication would make a world of difference—especially for those who are already dealing with severe fatigue, pain, and cognitive challenges. A once-daily chelation drug with fewer side effects would ease the burden on both patients and caregivers.