Wilson Disease; Available and Affordable Approaches to Treatment in Mississippi

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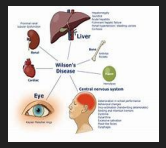
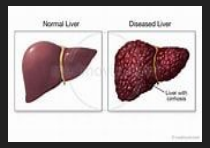
The Liver and Waste Removal

The liver is delicate and one of the most essential organs within the human body. The liver has 500 different functions, and one of its main processes involves the removal of waste, which includes excess copper, alcohol, and ammonia. If this waste is not removed, it can cause jaundice, liver failure, and eventually death. If the liver is malfunctioning, it could create a domino effect ranging from multiorgan failure to cirrhosis. In short, your liver is one of many organs that are your lifeline.[1] [2]





What is Wilson Disease?

Wilson disease is a rare genetic disorder that causes inability to excrete excess copper within the body. This leads to copper poisoning and further fatal illnesses, which is why early diagnosis is imperative. Diagnosing Wilson disease can be challenging, since symptoms often mimic other diseases and are often misdiagnosed. Onc test used to help make the Wilson diagnosis is via an ophthalmologic slit lamp examination for Kayser-Fleischer rings. These rings are brownish-yellow in color and appear around the corneo-scleral junction of the eye. They become more visible as the disease progresses, so they can be a good indication of the patient's condition.

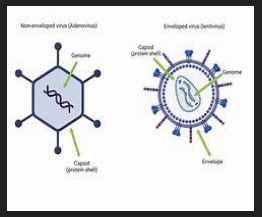
Signs of Wilson disease include a number of hepatic, neurological, and/or psychiatric symptoms. Cirrhosis, hepatitis, fatty liver, tremors, seizures, insomnia, depression, anxiety, and psychosis are all commonly present in patients. The first signs of Wilson disease in the body are often jaundice, stomach pain, vomiting of blood, and abdominal swelling. The excess of copper in the brain can lead to difficulty performing everyday tasks, such as walking, talking, writing, and swallowing food. Despite how quickly the disease progresses, Wilson will always be fatal if left untreated and requires a strict dietary and medication regimen for the duration of the patient's life. Therefore, it is vital that early treatment be made available for those affected by Wilson disease.[3]

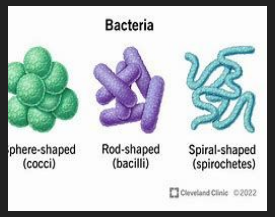
Treatments and Therapies for Wilson Patients

Wilson Disease has two well-documented and tested drugs that have shown life saving results. These treatments include zinc acetate and chelation therapy drugs. However, these drugs both have fatal flaws. The first refers to the life expectancy if the patient stops taking the drugs. They could die in 3 months if this occurs. This also correlates into the next flaw: affordability. Without any discounts or insurance, the drugs cost upwards of 300 dollars each month. This can be lethal in Mississippi, as the median salary is only $53,085. Most people in Mississippi do not have enough money to put back in case of a lay off, and if this period of no income persists, it could end in death. With this being considered, we think that a permanent treatment or cure is needed.

In vivo therapy could be the next step in creating a cure or permanent treatment. In vivo therapy is a virus injected from an IV into the body to correct a defective gene or chromosome. This is done by deactivating the virus and inserting the corrected gene into the defective gene. In vivo therapy could work because when a Wilson patient gets a liver transplant, the new liver would function as normal. This shows us that the gene defect ATP7B is local to the liver and if corrected, could be a cure to Wilson Disease.[4]

The human microbe biome is one of our most powerful tools in fighting diseases, so why should this stop at Wilson Disease? Genetically engineered bacterium or GEB might lead us into the next step of treating and possibly curing Wilson Disease. A GEB is defined as a bacteria that has the ability to express heterologous proteins or molecular compounds for a specific purpose after being genetically modified. We have created a proposal for possibly curing Wilson Disease. There is a bacterium that absorbs copper for metabolic processes being researched for getting rid of copper in mines and soil, it is called C. metallidurans. It absorbs copper ions via the CupA gene. Our idea involved giving a simple bacteria this gene and seeing how it reacts with the human microbe biome. This would take years, but this type of research is desperately needed for these rare diseases. Only 5% of rare diseases have a treatment, much less a cure.[5]





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About the Wilson Disease Association (WDA)

The Wilson Disease Association was founded in Binghamton, NY in 1978, initially with the intent to help two young boys who were recently diagnosed with the disease. Formally incorporated as a nonprofit in 1983, the organization consisted of a board of 7 members, all of whom either had Wilson disease or were relatives of someone who had been diagnosed. Ever since, the WDA has sought to "provide support and hope to people impacted by Wilson disease worldwide so that they may live the best quality of life possible."
One of the most significant research undertakings the WDA has assisted with is the Patient Registry Study. The Patient Registry Study aims to compile a vast array of information from patients across the United States, UK, Germany, and Denmark. This information is paramount in the development of clinical trials, treatments and healthcare policy regarding rare diseases. The WDA has contributed more than $2,470,000 to the project since its inception. The WDA also involves itself in workshops regarding Wilson disease and other diseases that affect copper metabolism, as well as programs assisting patients in underdeveloped nations who have little access to treatment.[6]

WILSON DISEASE ASSOCIATION

Policy Affecting Rare Disease Patients

Senate Bill 2156 (Blackwell)
Senate Bill 2156 (Blackwell) involves the creation of a Mississippi Rare Disease Advisory Council, also referred to as RDAC. This bill would affect every 1-10 Mississippians and 30 million people across America. It would create a council to collect and provide the data needed for lawmakers to make more informed decisions. This council would also help people who have Wilson Disease by providing more information to develop new treatments and plans of action and help save thousands of lives. This process continued for 2 years before ratification.[7]

Senate Bill 2858 (Lifesaving Treatment Act)
Eighty percent of rare diseases are genetic. The Lifesaving Treatment Act would allow patients individualized care based on genetic composition. This law was necessary because prior to its passing,, patients with rare diseases had to wait long periods of time for new treatments. Now that the law has passed, thousands of people can get treatment faster and prevent permanent damage. This law passed on July, 1st, 2024.[8]