An EASY way to become involved!

News about an exciting awareness and fundraising project for 2015! One of our members, Chris Simopolous from California, has come up with a powerful concept for a nationwide walk in the fall of 2015. If successful, we hope to make this an annual event! Here’s how it will work:

1. A Core Committee, chaired by Mr. Simopolous, will select a date, plan the execution and prepare a toolkit that will be rolled out to City Leads for local implementation.

2. The City Lead will receive support from the Core Committee, along with a toolkit outlining easy steps for creating a successful event. Once logistics are planned, the City Lead will be responsible for energizing friends and family to participate. No number is too small and none too big!

We will all walk on the same day, in various cities across the U.S., to raise awareness and funds for Wilson disease. The Core Committee has already been selected and is busy planning for this event. Think about having The BIG WOW in your city. You will be contacted by e-mail soon asking if you would like to be the City Lead in your city. If you are not on our e-mail list, and would like to be a City Lead, please contact membership@wilsonsdisease.org to let us know. The Committee promises to make your job fun and as easy as pie!

New Clinical Trial: Wilson Therapeutics Announces Start of Phase 2 Study to Evaluate the Efficacy and Safety of WTX101 in Newly Diagnosed Wilson Disease Patients

STOCKHOLM, November 17, 2014 (PR NEWSWIRE) – Wilson Therapeutics AB, a development stage biopharmaceutical company, today announced the start of a Phase 2 clinical study to evaluate efficacy and safety of its lead product candidate WTX101 in Wilson Disease, a rare genetic disorder and designated orphan disease, in which the body is unable to process copper.

“We are delighted to proceed with this important clinical study in Wilson Disease patients as a next step in the development program for WTX101,” said David Clark, MD, Chief Medical Officer at Wilson Therapeutics. “The data from this study will provide additional insights in the optimal dosing of WTX101, and further inform our planning for the Phase 3 program, which is currently in the final stages of clinical design.”

The phase 2 clinical trial will evaluate the efficacy and safety of WTX101 using an individualized dosing regimen in up to 30 newly-diagnosed patients with Wilson Disease. The study will be conducted at sites in the U.S. and Europe, and will follow patients on WTX101 for six months.

“We are very excited to offer people with Wilson Disease the opportunity to participate in the WTX101-201 study, the first clinical trial to evaluate a new therapy for Wilson Disease in this decade,” said Frederick Askari, M.D, Ph.D. Associate Professor and Director of the Wilson Disease Program and Wilson Disease Center of Excellence, Department of Internal Medicine, Division of Gastroenterology, at the University of Michigan. “Based on earlier clinical studies with tetrathiomolybdate in people with neurologic Wilson Disease, WTX101 holds great promise to mitigate the neurologic damage from Wilson Disease, so we are delighted it will be available for study participants as soon as possible.”

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The Wilson Disease Association is a nonprofit 501 (c)(3) organization. The Copper Connection is a semi-annual newsletter published by the Association that informs members of findings in the area of Wilson Disease. There is no copyright. Newsletters and other publications can disseminate any information in The Copper Connection. Please cite attribution to the Association and the author.

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Our Mission Statement
The Wilson Disease Association funds research and facilitates and promotes the identification, education, treatment and support of patients and other individuals affected by Wilson Disease.
New Clinical Trial
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Mary Graper, President of the Wilson Disease Association continued: “Although Wilson Disease has been recognized for more than one hundred years, it is still widely misunderstood. Because of this, many patients have a delayed diagnosis or a misdiagnosis causing disease progression, potentially irreversible organ damage, and even death. While there are currently several available treatments, there is a significant need for new treatment options such as WTX101 that could be of great benefit to patients with Wilson Disease.”

About WTX101
WTX101 is the proprietary bis-choline salt of tetrathiomolybdate, which has been evaluated for various indications in clinical studies involving over 500 patients, including 120 patients with Wilson Disease. Through its unique mechanism of action, tetrathiomolybdate has been shown to rapidly lower and control copper levels in both Wilson disease and other patients. Previous data also suggest that tetrathiomolybdate may stabilize neurological function and reduce the risk of neurological deterioration after initiation of treatment in Wilson Disease patients with neurological involvement. WTX101 has been shown to lower and maintain copper levels with once or twice daily oral dosing. WTX101 has received orphan drug designation in both the United States and the European Union.

All Centers Are Now Recruiting: Phase 2 Study in Newly Diagnosed Wilson Disease Patients with WTX101 (Tetrathiomolybdate)

Patients are being recruited for a Phase 2, multi-center, open-label, study to evaluate the efficacy and safety of WTX101 administered for 24 weeks in newly diagnosed Wilson Disease patients. This study is being sponsored by Wilson Therapeutics.

The study drug, WTX101 (bis-choline tetrathiomolybdate) is a de-coppering agent that is being investigated for the treatment of Wilson Disease. The aim of this study is to confirm that the dosing regimen planned for use in future studies with WTX101 is safe and effective in de-coppering newly diagnosed Wilson Disease patients. The study will be conducted at 6 Wilson Disease expert centers (University of Michigan Hospital, Ann Arbor, MI; Yale University Medical Center, New Haven, CT; UCLA Ronald Reagan Medical Center, Los Angeles, CA; Medical University of Vienna, Vienna, Austria; University Hospital, Heidelberg, Germany; Institute of Psychiatry and Neurology, Warsaw, Poland).

If you are a newly diagnosed Wilson Disease patient you may be eligible to join the trial that is currently underway, if you meet the following criteria:

• Male or female, aged 18 years or older
• Have elevated blood free copper levels
• Treated with chelation or zinc therapy for 28 days or less
• Have hepatic or neurological symptoms or both
• In otherwise general good health

The study lasts for 7 months. There are 8 study visits to the study site during this 7 month period and 5 additional study visits with a potential for a nurse to visit you at home to reduce the amount of travel for you. Wilson Therapeutics will pay for your travel expenses.

For further information about this study, please go to bit.ly/wtx101-201 for US clinical trial information or bit.ly/wtx101-201-eu for European clinical trial information. To discuss possible participation at any of the United States study centers please contact the University of Michigan Hospital Wilson Disease Clinic by calling 1-800-333-9013. Contact details for all the individual sites are listed in the clinical trials links above.
2014 WDA Annual Conference
By Carol Terry, WDA Secretary

Our 2014 Annual Conference was held on September 5-7 in Ann Arbor, Michigan. We had an excellent turnout and a very interesting slate of speakers. The weekend started with a welcome reception on Friday evening, where we had a chance to make new WD friends and renew old friendships. On Saturday morning, presentations were made by several members of the staff of the WDA Center of Excellence of the University of Michigan, including Center Director, Fred Askari, MD, PhD, Mathew Lorincz, MD, PhD, Emilie Klemptner, MS, RD, and Patricia Paulin, RN. Dr. Askari gave us a preview of WD research and care in the future, while Dr. Lorincz presented an overview of the neurological manifestations of WD. Ms. Klemptner and Ms. Paulin discussed low copper diet.

In addition, Eva Margot Kant, LCSW-R, from Hunter College and Columbia University, gave us some rousing insights on how to communicate effectively with your doctor or other healthcare professional.

After lunch, we heard about the outlook for gene therapy for Wilson disease from Olivier Danos, PhD, of Kadmon Corporation, and the current program for molecular genetic testing of WD at University of Michigan from Marwan K. Tayeh, PhD. We also heard about the development of Gluzin (zinc gluconate) from John WU, President and CEO of extreme. The remainder of the afternoon was spent in a number of breakout sessions to give attendees a chance to have some give and take about issues of interest.

Saturday night’s conference banquet was highlighted by a keynote address by David Clark, MD, CMO of Wilson Therapeutics. Dr. Clark outlined the plan for clinical trials and development of WTX101, a new treatment for WD. WTX101 is a new version of tetraethylthiometabolite (TM), and clinical trials will begin later this year, initially in newly diagnosed WD patients.

Sunday morning we had a special treat—breakfast with Dr. George Brewer. As you may know, Dr. Brewer is responsible for the development of zinc acetate (Galzin) as a maintenance treatment for WD. In his presentation, Dr. Brewer gave us some interesting thoughts on zinc, copper, Wilson disease, and Alzheimer’s disease.

9th International Copper Meeting
By Mary Graper, WDA President

The 9th International Copper Meeting (Copper 2014) took place in Vico Equense, Italy October 5th – October 10th 2014 at the Aequa Hotel. The Copper meeting is held every other year, always somewhere in Italy. It brings together leading investigators in the fields of biology, chemistry, medicine, and the environment. Their research aims are the same; how copper, too much or too little, affects life.

I had the opportunity to attend part of this wonderful meeting while traveling on a personal vacation with my husband. It was wonderful to see some familiar faces and meet some new who are so dedicated to the “copper cause”.

Vico Equense is a beautiful town just outside of Naples and sitting on a bluff above the coast of the Mediterranean Sea. The meeting organizers could not have picked a better location. One of the meeting organizers was WDA Medical Advisor, Dr. Dennis Thiele from Duke University. Another WDA Medical Advisor, Dr. Svetlana Lutsenko from Johns Hopkins in Baltimore, gave a presentation about her laboratory’s recent research. Her topic was Activation of Nuclear Receptors Improves Liver Morphology and Function in Wilson disease. This study follows a series of earlier studies, which reported that when copper accumulates in the liver it changes levels of cholesterol and fat. [Annals of the New York Academy of Sciences, Volume 1315, Human Disorders of Copper Metabolism II pages 56–63, May 2014] continued on next page
Copper Meeting
continued from previous

While this meeting focused on basic laboratory research, I think it is important to appreciate how this work ultimately increases our understanding of how to best treat patients with copper disorders. Without “bench” research “bedside” treatment would not be possible. A good example of this is Dr. Lutsenko’s research, which currently focuses on using Wilson disease mouse models. The hope is that the treatment can be adapted to human patients in the future.

Disability Pride, September 20th, 2014
Carol McCullough, WDA member & Director-Northern Ireland Rare Disease Partnership

In September of this year Belfast hosted the UK’s first ever Disability Pride parade. We had anticipated that this was going to be a very special day and excitement grew as we arrived in Writer’s Square for the beginning of the carnival. Rui Alves from Portugal had designed a poster for WDA and one of my relative’s pasted it onto a placard for the day. Fortunately most of the charities had their own placards which made it easier to find the rare disease charities that were to follow the Northern Ireland Rare Disease banner. Then it was 11am and we were off. A lot of folk had dressed up in costumes; there were floats; there was music, singing and dancing; a mass of colour - a long procession of people who have disabilities, their families, friends and supporters. In the true spirit of Disability Pride there was much laughter. It was all very special for me. We were walking with other rare disease charities to demonstrate that cumulatively rare diseases are not rare, should never be forgotten conditions, and that we are “Stronger Together”; and never in my wildest dreams could I have imagined ‘the name Wilson’s disease’ being carried past the throng of people who lined Belfast’s Royal Avenue. An awesome experience.

Disability Pride in Chicago chose Belfast for this year’s carnival because they were so impressed by Michael Holden’s efforts to improve services for people with disabilities. After we all had arrived at the City Hall Michael, who has Motor Neurone Disease, made a short speech and the next stage of celebrations began. There were marquees to visit, including NIRDP’s marquee; there was food and entertainment. We heard Skyzdalimit Omagh a cross-community performing arts charity for people with a learning or physical disability. Ska band, “Doghouse “and pop band Saffyre. We watched the talented Strictly Wheels a Strictly Wheelchair Dancing Duo; and Gary and Paula who reached the semi-finals of Britain’s Got Talent in 2012. People passing by gathered to find out what was happening and stayed. A camera scanned the crowd throughout the day and the party atmosphere was captured on City Hall’s Big Screen. The weather was that of a glorious summer day in late September; definitely a T-shirt day that no one had expected.

Disability Pride Belfast carried on until 5pm. Thousands had gathered to celebrate the contribution of people who have disabilities and to respect those who have disabilities. It was nothing less than brilliant- all of it. We want one every year.
Thanks for your support

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IMPORTANT NOTE:
Recently we discovered a loss of data in our membership database.
This consisted of donations that were received between January 2013 and June 2014. In the process of recovering this data, we discovered that many donations were never acknowledged by the WDA. We have rectified this to the best of our ability.

However, if you sent a donation for which you never received a thank you, please e-mail us at membership@wilsonsdisease.org to let us know. We will happily resolve this for you as quickly as possible.

For your convenience, you can make your donations online by visiting our website. Just visit www.wilsonsdisease.org you will see the Donate button on the front page. This will direct you to a secure PayPal link where you can safely enter your information. We encourage you to donate online as it will save you time and postage! If you prefer to mail a check, or do not have online access, please send your tax-deductible donation to the WDA office at:

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