Medicare Coverage of Molecular Pathology Procedures

Last year Palmetto GBA, who has administered Medicare health insurance for the Centers for Medicare & Medicaid Services (CMS) throughout the US and its territories since the inception of the program in 1966, made the decision not to cover molecular genetic testing for the diagnosis of Wilson disease and many other diseases. About coverage of testing for Wilson disease, they state:

“ATP7B gene mutations have been primarily associated with Wilson Disease, a disorder of copper metabolism. However, serology remains the gold standard for testing and treating the signs and symptoms of this condition. At present, the literature does not support that ATP7B gene testing changes physician treatment or improves patient outcomes. Therefore, Palmetto GBA has determined ATP7B gene testing is a statutorily excluded service. Palmetto GBA will also deny panels of tests that include the ATP7B gene.”

Medicare will not pay for the genetic test. We feel strongly that Palmetto’s statement is incorrect. There are known cases where Wilson disease could not be diagnosed by serology [blood tests] alone. In fact, the AASLD Practice Guidelines, Diagnosis and Treatment of Wilson Disease: An Update continued on page 7

New Drug Under Development for Wilson Disease

VWTX101 is a development stage therapy for Wilson Disease that is advancing towards late-stage clinical trials. WTX101 is the proprietary bis-choline salt of tetrathiomolybdate (TTM). TTM is a novel de-coppering agent with a unique mechanism of action that has demonstrated a more rapid and improved control of copper in Wilson Disease patients.

Unlike other de-coppering agents currently available for the treatment of Wilson Disease that form unstable complexes with copper and other metals (e.g., iron and zinc) and are excreted via urine, TTM selectively forms high stability complexes with copper and proteins. These complexes are then primarily excreted via the bile, restoring the normal excretion continued on page 5

$13,000 Raised to Aid Midlothian Man Suffering from Rare Genetic Disease

SEES Student Coin Challenge Raises $7,400+ in a Week

RICHMOND, Va.— The Michaux family of Midlothian is waging war. They call it “Woody’s War on Wilson’s” and it’s a battle for their 22-year-old son, Woody Michaux. Woody was a typical college student studying chemistry and physics at Hampden-Sydney College in Farmville, Va., until two years ago when he was diagnosed with Wilson’s Disease, a rare genetic disorder. The disease has caused severe neurological symptoms due to his body’s inability to rid itself of copper, resulting in it accumulating in his brain. Since his diagnosis, medication is helping, but Woody still requires round-the-clock care with feeding, personal care, medications and therapy. continued on page 2

Mrs. Mooney and Woody
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NEWSLETTER DEADLINE:
If you would like to submit an article to be published in the next printed edition of The Copper Connection the deadline for submission is September 1, 2014. Please e-mail your article to the WDA office at info@wilsonsdisease.org

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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www.wilsonsdisease.org

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.
Midlothian Man
continued from page 1

Woody and his family are also in need of a new, permanent ramp for their house and a wheelchair-accessible restroom. Currently, Woody must be taken to a nearby gym to bathe. When Woody’s former fifth grade teachers, Michelle Mooney and Krista Stackow, at St. Edward-Epiphany Catholic School (SEES) learned of this, they immediately adopted his cause as theirs.

This school year, the SEES fifth grade has conducted several fundraising activities for Woody’s cause. Their most successful project to date was completed Friday (Jan. 17, 2014) with a school-wide, week-long coin drive. The goal was to raise $5,000 in loose coins. Last week, SEES students and faculty raised a remarkable $7,445.80. Student and parent volunteers counted and rolled 57,591 coins. The coin drive combined in-kind donations and the fifth grade’s bake sale and waffle breakfast have raised more than $13,000 for Woody’s cause. For more details, click on http://www.gofundme.com/woody.

“Our students and staff have an incredible capacity for helping others,” said Emily Elliott, SEES principal. “Woody and his family are very much a part of the St. Edward-Epiphany School family, and it’s been a blessed privilege for us to have this opportunity to make a hopeful difference in their lives.”


Patient Assistance for Cuprimine, Syprine and Galzin

Patient Access Network (PAN) Foundation is dedicated to providing help and hope to underinsured patients who would otherwise be unable to afford high-cost specialty medications. PAN provides assistance through nearly 60 disease-specific programs designed to help patients being treated for certain cancers, chronic illnesses, and rare diseases. Since 2004, PAN has provided nearly $400 million in financial assistance to more than 200,000 patients. Through a quick, streamlined application process for patients and easy-to-navigate online portals for specialty pharmacies and providers, PAN makes it simple and convenient for patients to get the financial assistance they need quickly, so they can initiate treatment immediately.

PAN’s Wilson Disease Assistance Program provides qualifying patients with $10,000 per year to use toward their out-of-pocket costs associated with their medications such as co-pays and coinsurance. To qualify, patients must have some form of insurance that covers the medication for which they are seeking assistance, must reside and receive treatment in the United States, and must have a household income at or below 500% of the Federal Poverty Level ($78,650 for a family of two). To learn more, visit www.PANfoundation.org/wilson-disease.
Rare Disease Day 2014 Joint North South Ireland Event, Riddle Hall, Belfast

By Carol McCullough, WDA Member, Armagh Northern Ireland; Board of Directors-Northern Ireland Rare Disease Partnership (NIRDP)

On 28th February over 200 people from around Ireland gathered in the Riddle Hall, Belfast to celebrate international Rare Disease Day 2014. ‘Celebrate’ is the most fitting description. We were reminded of progress made for improving the plan for rare diseases over the previous twelve months; and progress made throughout Europe over the past ten years. We celebrated two Health Ministers’ confirmed intention to release the finalised rare disease implementation plans in 2014. We listened as health care professionals and other academics explicated the utility of registries, of on-going developments in the area of electronic health records and patient healthcare passports. We listened to patient and carers’ experiences: what living with rare disease is really like, what is important, how linking up helps. We also heard very moving patient experiences from the floor. We mingled; meeting old friends and making new ones. We spoke of our own experiences.

This year’s theme for Rare Disease Day was ‘Joining Together For Better Care’. It was an opportunity for me to join up with Karen; only the second person I have met outside my own family who has been diagnosed with Wilson’s disease. However we belong to a much larger extended family, we have discovered the impact of “Stronger Together” and of how much can be achieved through working within that extended family. Yet small things matter.

The WDA banner, standing proud beside other voluntary organisation’s banners, draws attention. Voluntary group leaflets are a source of information. When one person stops to ask “What is Wilson’s disease” the fact that one more person leaves knowing something about Wilson’s disease is an achievement. In the same way making the most of opportunities such as medical appointments can be achievements. Passing professionals a new article on Wilson’s disease, providing them with links to international guidelines, drawing attention to the new UK Strategy for Rare Diseases, asking a Neurologist if he would be happy to put an A4 size version of the WDA poster on his ‘dedicated’ wall space, or giving a Practice Nurse a patient healthcare passport for consideration of its value are achievements.

We can all become stronger together by standing behind our individual rare disease support groups. We have discovered that “Stronger Together” works; and whatever we do, whether it be a major piece of work or the small things that add up, it has had an impact. Rare Disease Day is our day to celebrate because we know how far that we have come.
### WDA Ribbon

Here they are! Ribbon designed and donated by Sarah Hendrix.
Copper metallic, with blue and white enamel image of WDA globe, lapel pin through the middle.

$7.00 suggested donation per ribbon (S & H included)

<table>
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<tr>
<th>Number of Ribbons</th>
<th>Amount $</th>
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### WDA Awareness Bracelet

Good news! A brand new shipment of these popular bracelets has just arrived.

Added to the new design is an image of the WDA globe as shown in the picture. The bracelets are packaged in groups of 5. The suggested donation per bracelet is $2.50, that comes to $12.50/package.

Orange/yellow swirl design, inscription reads “STOP COPPER!” and “www.wilsonsdisease.org”.

<table>
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<tr>
<th>Packages of Bracelets</th>
<th>Amount $</th>
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### WDA Lapel Pin

Copper metallic edge and figures, with blue and white enamel background. On-half inch in diameter.

Suggested donation is $5.00 each, or 5 for $20.00

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<th>Number of pins</th>
<th>Amount $</th>
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### The Gift of Life 2

This 382 page book is a valuable resource for anyone facing a liver transplant. Co-author and former WDA Board member, Parichehr Yomtoob, has generously donated a number of copies to benefit the WDA.

Mrs. Yomtoob’s son David was a Wilson disease patient who underwent 3 liver transplants during his lifetime. Sadly, David passed away following his third transplant in 2006.

Suggested donation is $22.00.

<table>
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<th>Number of copies</th>
<th>Amount $</th>
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Displayed below is the ballot that will be used for elections at the Annual Conference in Ann Arbor. If you will not be attending the conference and are a current member of the WDA, you may vote by mailing this ballot to the WDA office before the Conference.

### Wilson Disease Association International 2014 Election Ballot

#### I. Nominee for re-election to Board of Directors (3-year term):

- **Patricia Paulin** – Ms. Paulin is a Registered Nurse and has worked as the patient contact at the Wilson Disease Clinic at the WDA Center of Excellence at the University of Michigan for over 11 years. She also made presentations at a past WDA Annual Conference and the 2013 Scientific Workshop held at Johns Hopkins University. Patti has served an initial one-year term as a member of the Board.
Membership Form

☐ New Member  ☐ Renewing Member  ☐ Mr.  ☐ Mrs.  ☐ Miss  ☐ Ms.  ☐ Dr.  ☐ Other

Name
Address__________________________ State_________ Zip__________ Country______________
City__________________________ Work Phone______________ Email____________________

☐ Membership Dues: Basic Membership - $35
  • Receive quarterly WDA Newsletter via e-mail – Please provide e-mail address above

☐ Basic Plus Membership - $60
  • Receive quarterly WDA Newsletter via U.S. mail or e-mail – Please provide email address above

☐ Silver Membership - $120
  • Receive quarterly WDA Newsletter via U.S. mail or e-mail – Please provide email address above
  • 10% discount on Annual Meeting registration for up to two registrants

☐ Gold Membership - $250
  • Receive quarterly WDA Newsletter via U.S. mail or e-mail - Please provide email address above
  • 15% discount on Annual Meeting registration for up to four registrants
  • Special recognition as a Gold Member in WDA Newsletter

☐ Copper Membership - $1000
  • Receive quarterly WDA Newsletter via U.S. mail or e-mail – Please provide email address above
  • 20% discount on Annual Meeting registration for up to four registrants
  • Special recognition as a Copper Member in WDA Newsletter
  • 10% discount for Annual Meeting program ad
  • No annual dues

I wish to make a donation to Wilson Disease Association:
Name__________________________ State_________ Country______________ Zip__________

☐ I am making a tax-deductible donation of $__________
☐ In honor of______________________ ☐ In memory of________________________
Send acknowledgement to: ______________________________________________________
Name__________________________ Street__________________________
City__________________________ State_________ Zip__________ Country______________

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Payment Information:
Marketplace Membership Fee Donation Total
$_____ $_____ $_____ $_____
☐Visa ☐ Master Card ☐ Check or Money Order attached
Card # ____________________________
Expiration Date:__________ CID # ________ (3 digits on back of card)

Please mail, tax, or e-mail to:
Wilson Disease Association, 5572 North Diversey Blvd., Milwaukee, WI 53217
Fax: 414-962-3886  E-Mail: membership@wilsonsdisease.org
New Drug
continued from page 1

route of copper that is impaired in patients with Wilson Disease. By rapidly binding and controlling copper in stable complexes, TTM may reduce the risk of mobilizing and transiently increasing the levels of free copper in patients starting de-coppering therapy. The rapid and improved control of copper is very important as high levels of copper cause tissue damage and transient increases in free copper after initiation of therapy is believed to be involved in causing additional tissue damage, especially in the central nervous system.

As a result of the improved control of copper WTX101 is therefore expected to improve control of the disease as well as reduce the risk of neurological deterioration after initiation of treatment in Wilson Disease patients with neurological disease.

The improved salt formulation of TTM, WTX101, has also been tested in clinical trials in oncology and was found to be safe and tolerable while efficiently lowering copper levels with once daily dosing. A once daily dosing regimen is expected to translate into improved patient compliance in Wilson Disease patients and therefore fewer treatment failures.

TTM has been tested in over 500 patients for up to 7 years, primarily in oncology and Wilson Disease, and Wilson Therapeutics is now preparing to advance WTX101 into late-stage clinical trials in patients with Wilson Disease.

Wilson Therapeutics is committed to advancing WTX101 through clinical trials with the aim of helping to improve the lives of patients with Wilson Disease. For more information, including patient stories, visit the Wilson Therapeutics website at www.wilsontherapeutics.com

Company Fundraiser in Honor of Kelvin Scharf

Mr. Kelvin Scharf of Clifton Park, New York was being treated for Parkinson disease since 2007. The treatment was not working. In 2009 Mrs. Scharf contacted the WDA for a referral to a Wilson disease physician in the area. The WDA directed her to Dr. Michael Schilsky, at Yale University, who then properly diagnosed Kelvin with Wilson disease in 2010.

Mr. Scharf had been Vice President of Finance at Hill & Markes in Amsterdam, New York until he was forced to retire in 2012 due to disabilities caused by Wilson disease. Hill and Markes recently spent a month doing various fundraisers for the WDA in recognition of Kelvin Scharf’s achievements at Hill & Markes. In a letter to the WDA, a Hill & Markes representative stated, “Kelvin has mentored many employees in our organization. He is a person who has helped to build this business over the years he has been with our company. On Friday, January 10, 2014, the company had a luncheon to talk about Kelvin, and his contributions to the company and presented [a generous check] of company matched funds to the Wilson Foundation (WDA).”

Editor’s note: Mr. Scharf passed away unexpectedly on April 16, 2014.

Trientine Crisis in Canada

Eve A. Roberts, MD, MA, FRCP, University of Toronto, Division of Gastroenterology, Hepatology and Nutrition, The Hospital for Sick Children, Toronto, Ontario

In the early 2000s we worried that cost of D-penicillamine (Cuprimine) and trientine (Syprine) would rise when Merck sold its licenses for those drugs, which are vital to the well-being of Wilson disease patients, to Aton Pharmaceuticals. Prices for both drugs drifted up somewhat. In 2010 Valeant acquired Aton and, of course, its licenses with that purchase. Prices again rose. In Canada this was problematic because trientine is a true orphan drug in Canada, and Syprine does not have the regulatory status to be covered by third parties such as public drug benefit plans or private drug insurance. Thus in most cases (there are exceptions) patients have to pay for trientine out of their own pocket. However, not everyone bought his/her Syprine because in 2010 Valeant Canada also established a compassionate provision programme, whereby Valeant Canada supplied Syprine free to them. In October 2013 Valeant Canada (not the WDA) decided to terminate this free programme as of January 1, 2014. At the same time they announced that Syprine would be sold in Canada at the Canadian equivalent of the price in the USA. This put the price in the range of at least CDN$11,000 per bottle of 100 pills. This price is unattainable for Canadian patients. Through a complicated process of advocacy and consultation, an alternative to Syprine is being sought. Canadian specialists who treat Wilson disease can contact the WDA to find out what to do right now. This situation is still in flux; however, progress is being made. Stay tuned!
WDA Conference 2013 in Los Angeles

Jeanne Friedman, WDA Board member

As usual, the conference kept us busy, with presenters speaking to us, showing slides and holding panel discussions, yet there was also some time for networking and opportunities for patients and family members to meet each other and share experiences and contact info. We met Dr. Jeff Bronstein, Director of the WD Center of Excellence and Director of the Movement Disorders Program at UCLA and were impressed by his knowledge of WD and by his willingness continue to learn even more from his patients. We heard from Dr. Fred Askari, Director of WD Center of Excellence at the University of Michigan, now a very familiar face and voice at these conferences. We heard from Dr. Hugh Young Rienhoff, Jr. from the Children’s Hospital Oakland Research Institute in Oakland, CA., about his own experience dealing with the challenge of getting his daughter diagnosed with her own rare disease. We heard from Dr. Allan Wu, about treatment of dystonia at UCLA and from Dr. Maren T. Scheuner who spoke about genetic counseling (at UCLA). Dana Hunnes, PhD, MPH, RD, spoke on the popular subject of diet and WD.

The breakout sessions were appreciated and well attended by the clinicians as well as by WD patients and families. A sign of a good conference is when people actually stay to the end; that we had people just hanging out to continue to share with each other and learn from each other beyond the scheduled ending of the conference speaks to it’s success.

All in all, we had interesting presentations and a nice-sized group of attendees, all of whom seemed happy they had come. So often this disease leaves us feeling helpless and very much alone. These conferences provide a wonderful opportunity to learn and share and feel part of a bigger movement toward greater understanding and better health. I’m eagerly looking forward to the next one.

SAVE-THE-DATE!

The WDA Annual Conference, held in conjunction with the University of Michigan, will be September 5-7, 2014 at the Holiday Inn (near the University of Michigan)
3600 Plymouth Road
Ann Arbor, MI 48105
Medicare Coverage
continued from page 1

http://bit.ly/L8QMUF, clearly state, “Mutation analysis by whole-gene sequencing is possible and should be performed on individuals in whom the diagnosis is difficult to establish by clinical and biochemical testing.”

The WDA, represented by Mary Graper, has participated in two conference calls with CMS along with representatives from the following organizations: American College of Medical Genetics and Genomics, American Society of Clinical Laboratory Science, American Society for Clinical Pathology, American Society for Histocompatibility and Immunogenetics (ASHI), Association for Molecular Pathologists, College of American Pathologists, and the National Fragile X Foundation. Through our work, we hope to effect change to the statutory exclusion for the top 100 diseases for which genetic testing is not covered by Medicare.

Thanks for your support

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Rick Wade - Walter and Lori Wall

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Wilson Disease Association
5572 N. Diversey Blvd.
Milwaukee, WI. 53217
TO:

Wilson Disease Association International
Annual Conference

SAVE-THE-DATE
September 5 - 7, 2014

Holiday Inn
(Near the University of Michigan)
3600 Plymouth Road
Ann Arbor, MI 48105