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Icahn School of Medicine at Mount Sinai and Bene Pharmachem GmbH To Collaborate On Clinical Studies For Mucopolysaccharidoses

NEW YORK—May 15, 2013/Press Release/-- The Icahn School of Medicine at Mount Sinai [[link http://icahn.mssm.edu/](http://icahn.mssm.edu/)] has signed an agreement with bene pharmaChem GmbH [[link http://www.bene-pharmachem.de/index.php](http://www.bene-pharmachem.de/index.php)] to collaborate to conduct clinical studies of pentosan polysulfate (PPS) in patients with mucopolysaccharidoses (MPS). The company, based in Germany, is the sole producer of pharmaceutical pentosan polysulfate.

The announcement was made on Wednesday, May 15, which is International MPS Awareness Day [http://www.impsn.org/page/imps_day]. MPS are a group of inherited metabolic diseases in which a defective or missing enzyme causes large amounts of complex sugar molecules to accumulate in harmful amounts in the body's cells and tissues. This accumulation causes permanent, progressive cellular damage that affects appearance, physical abilities, organ and system functioning, and, in most cases, mental development. Currently there is no cure for these disease syndromes. Medical care is directed at treating systemic conditions and improving the person's quality of life. (To learn more: <http://www.ninds.nih.gov/disorders/mucopolysaccharidoses/mucopolysaccharidoses.htm>).

The original discoveries demonstrating the utility of **(PPS)** for the treatment of MPS and other lysosomal storage diseases were made in the Mount Sinai laboratories of Calogera M. Simonaro, MPh, PhD, Associate Professor of Genetics and Genomic Sciences and Edward H. Schuchman, MPh, PhD, Professor of Genetics and Genomic Sciences. Dr. Simonaro and Dr. Schuchman have demonstrated in MPS animal models that PPS has the effect of reducing the chronic inflammation associated with substrate accumulation, resulting in improved motility and a slowing of bone/cartilage disease. These effects were superior to other anti-inflammatory drugs such as anti-TNF alpha antibody therapy. Their study, **“Pentosan Polysulfate: A Novel Therapy for the Mucopolysaccharidoses,”** was published by *PLOSOne* on January 24, 2013 (<http://www.plosone.org/article/info%3Adoi%2F10.1371%2Fjournal.pone.0054459>).

The agreement between Mount Sinai and bene also includes a supply of PPS for the clinical trials and licensing arrangement to support approval and commercialization of PPS for MPS patients.

"We are excited by the potential of pentosan polysulfate (PPS) for the treatment of MPS and other lysosomal storage disorders," said Dr. Simonaro and Dr. Schuchman. "Our primary goal has been to evaluate the safety and effectiveness of this drug, first in MPS animal models, and now, in a collaboration with bene, in clinical trials in MPS patients. We are very pleased that bene will collaborate with us to test PPS for MPS in a formal clinical trial setting, so that the therapy can be approved and available for use by all patients. We will continue to inform the MPS patient community of our efforts."

"We are encouraged by this agreement between Mount Sinai and bene, which has the potential to benefit patients with MPS and their families," said Barbara Wedehase, Executive Director of the National MPS

Society [<http://www.mpsociety.org/>]. “We are long-time supporters of Dr. Simonaro’s research and look forward to the results of this collaboration.”

Mount Sinai and bene are working with leading MPS clinicians to design an appropriate clinical trial to evaluate the safety and efficacy of PPS. Dr. Simonaro and Dr. Schuchman are working to identify an optimal administration of PPS for clinical study in MPS patients. Ongoing studies include an assessment of the pharmacokinetic, biodistribution and toxicological properties of pentosan polysulfate in the specific context of MPS disease, as well as identifying the optimal formulation.

The Ryan Foundation for Orphan Disease Research [<https://www.facebook.com/pages/The-Ryan-Foundation-for-Orphan-Disease-Research/275752862497399?sk=info>] will be funding an initial study on MPS I, and anticipates starting to enroll MPS patients later this year. "We have been following this research from Dr. Simonaro's presentations at WORLD and the National MPS Society Conference, and are aware of Mount Sinai's progress with PPS as it applies to MPS I. We look forward to this exciting collaboration, and helping people," states Mark Dant, President of The Ryan Foundation (on Facebook at The Ryan Foundation for Orphan Disease Research.)

About The Mount Sinai Medical Center

The Mount Sinai Medical Center encompasses both The Mount Sinai Hospital and Icahn School of Medicine at Mount Sinai. Established in 1968, the Icahn School of Medicine at Mount Sinai is one of the leading medical schools in the United States. The Icahn School of Medicine is noted for innovation in education, biomedical research, clinical care delivery, and local and global community service. It has more than 3,400 faculty members in 32 departments and 14 research institutes, and ranks among the top 20 medical schools both in National Institutes of Health (NIH) funding and by U.S. News & World Report.

The Mount Sinai Hospital, founded in 1852, is a 1,171-bed tertiary- and quaternary-care teaching facility and one of the nation’s oldest, largest and most-respected voluntary hospitals. In 2012, U.S. News & World Report ranked The Mount Sinai Hospital 14th on its elite Honor Roll of the nation’s top hospitals based on reputation, safety, and other patient-care factors. Mount Sinai is one of just 12 integrated academic medical centers whose medical school ranks among the top 20 in NIH funding and by U.S. News & World Report and whose hospital is on the U.S. News & World Report Honor Roll. Nearly 60,000 people were treated at Mount Sinai as inpatients last year, and approximately 560,000 outpatient visits took place.

For more information, visit <http://www.mountsinai.org>.

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About bene pharmaChem

bene pharmaChem is a manufacturer and supplier of high quality products for medication and represents the highest degree of technological competence in the production of pharmaceutical ingredients. bene pharmaChem combines state-of-the-art research and development with long-standing medical and pharmaceutical expertise supported by a global network of external partners. bene pharmaChem is known for its investment in the future of medicine and for its concern in accepting social and ecological responsibilities.

bene pharmaChem is a member of the bene corporation and has been owned by the Benend family for two generations. Since its foundation in 1949 bene’s name has come to represent innovation and high-

quality in the manufacture of medicines. The foundation for bene's success today was laid some 50 years ago with the development of pentosan polysulfate sodium and ben-u-ron. Both established bene's reputation as a manufacturer of high-quality medicines and they are still marketed successfully today. For more information, visit <http://www.bene-pharmachem.de/en/company.php>.