New therapy option for nerve disorder MLD

12/01/2021 – PROvendis to present technologies from NRW at BioFIT Digital 2021.

The massive damage to the nervous system involves the progressive loss of motor and mental abilities.
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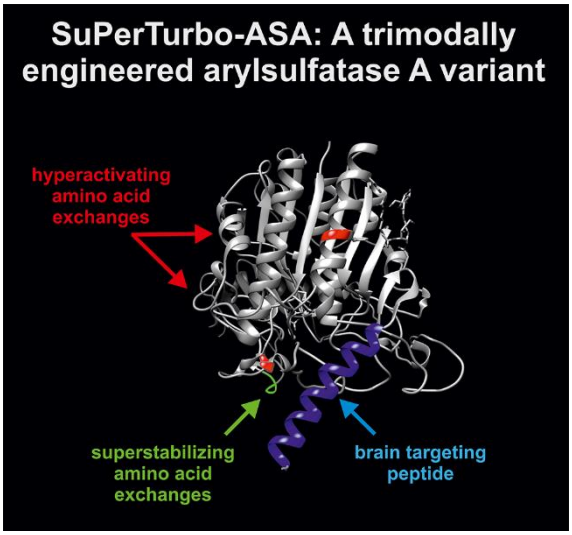
Metachromatic leukodystrophy (MLD) is a rare hereditary disorder of the nervous system in which the buildup of sulfatides destroys the protective myelin sheath around the nerves, resulting in serious neurological problems that eventually lead to death. It is caused by a deficiency of the enzyme arylsulfatase A (ASA), preventing the breakdown of sulfatides. The research team around Dr. Ulrich Matzner of the University of Bonn has been able to modify ASA, so it is much more active and stable than the normally occurring enzyme, human wild-type ASA. A third modification involves more efficient penetration of the blood–brain barrier. PROvendis will be pitching these and other inventions in clinical diagnostics and drug development from higher-education institutions in North Rhine-Westphalia at BioFIT Digital 2021 on December 9th.

More efficient enzyme transfer reduces destruction of myelin sheath



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A genetic defect in MLD patients causes the malformation of the ASA enzyme. Based on the enzyme variant developed and patent pending, it is now possible to infuse or inject patients with this hyperactive and highly stable enzyme, a procedure called enzyme replacement therapy. The ASA enzyme travels through the bloodstream to its targets, the brain and spinal cord. The breakdown of the sulfatides prevents buildup on the myelin sheath of nerve cells and, with it, the destruction of myelin-forming cells and nerves. *In vivo* experiments using an MLD mouse model showed a therapeutic efficiency eight times higher than the human wild-type ASA. After four treatments, sulfatide deposits were reduced by over 60 percent.

Earlier clinical trials with the human wild-type ASA enzyme demonstrated the viability of an enzyme replacement therapy, however, the therapeutic effects of the low dose of the enzyme used were minor. A higher dose was contraindicated due to the potentially serious immunological side effects. The benefit of the genetically modified enzyme is that the dose can be significantly reduced, and it can get into the brain.

Major industry gathering for innovations in the life sciences

As one of the leading events for innovations from European life sciences research, BioFIT invites researchers, technology transfer experts, and representatives of the pharmaceutical and biotech industry to get together every year to exchange knowledge. Research results, especially in the area of innovative treatment methods for patients, are presented to interested businesses and in order to clinical use. At BioFIT Digital 2021, Dr. Jürgen Walkenhorst from PROvendis, on behalf of NRW Hochschul-IP, the association for Intellectual Property of 28 higher-education institutions in North-Rhine Westphalia, will be presenting the latest in life science technologies from North Rhine-Westphalia. He will also be moderating a panel on *How to collaborate on academic research assets such as databases and registries?*

Our technology offering on this novel MLD therapy can be found here.

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About PROvendis GmbH

PROvendis acts as a professional service provider in the entire field of IP management for more than 30 universities and extra-university research institutions as well as for companies and start-ups.

NRW Hochschul-IP — Network for Intellectual Property (IP) of NRW Universities

28 universities of North Rhine-Westfalia and PROvendis GmbH form the network NRW Hochschul-IP. The network for Intellectual Property (IP) encourages professional knowledge and technology transfer. Together with the University of Münster (WWU) PROvendis acts as the central service provider for NRW Hochschul-IP. The network NRW Hochschul-IP is funded by the federal state of North Rhine-Westfalia. Grant authority is the Ministry of Economic Affairs, Innovation, Digitalization and Energy of the State of North Rhine-Westphalia.