



*A Philanthropic and Investment Alliance turning science into medicines*

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**BioPontis Alliance for Rare Diseases announces agreement  
with Massachusetts General Hospital on new treatments for Huntington's disease.**

Raleigh, NC, USA; Brussels, Belgium; and Boston, MA, USA.

Today, BioPontis Alliance for Rare Diseases, an international nonprofit organization and Massachusetts General Hospital (MGH), the largest hospital-based research program in the United States, announce an agreement initially targeting the treatment of Huntington's disease. The mission of BioPontis Alliance is to advance promising basic research into treatments for rare neurologic diseases. To broaden the funding basis of the mission, BioPontis Alliance has also established an impact investment portfolio subsidiary. The strategic collaboration between MGH and BioPontis Alliance will advance discovery research being conducted at MGH, Massachusetts Institute of Technology (MIT), and Northwestern University on sirtuin 2 deacetylase inhibitors to pre-clinical testing in order to develop a potential treatment of neurological disorders like Huntington's disease.

Huntington's disease is a progressive genetic degenerative brain disorder that causes uncontrolled movements, emotional problems, and loss of thinking ability (cognition) and ultimately death. There are no therapies available for Huntington's disease patients today.

The agreement establishes a pathway for the joint development of basic research toward a treatment candidate. It joins the basic research and early stage translation resources at MGH with the rare disease therapeutics development expertise at BioPontis Alliance. "As clinicians and scientists, we are committed to not only unravel the basic mechanisms of diseases like Huntington's disease, but to see this basic research fuel discovery of new therapeutics. This is a unique opportunity for us, to collaborate with BioPontis on one of the major long term aims of our research, namely translating ground-breaking basic findings into treatment opportunities for patients", commented Anne Young MD, PhD, and former Chief of Neurology at MGH.

The Huntington's disease patients' organizations are pleased to see BioPontis Alliance starting this development project with MGH researchers. "The European Huntington Association (EHA) strongly endorses this collaboration. We recognize the need to support academic results to reach further towards the clinic and BioPontis' effort to bridge this gap in rare diseases is highly appreciated. This early stage research combined with the expertise at BioPontis Alliance provides additional hope to our patients, and their families that a therapy will be developed for their disease. The strategy of BioPontis to integrate the patients' perspective as early as possible in the process we believe will benefit the projects and hopefully contribute to success.", states Astri Arnesen, President EHA.

BioPontis Alliance is working to identify and develop a portfolio of new candidate medicines for rare diseases where there is no current treatment available. We will realize this with the support of our philanthropic donors, and with the investment in our portfolio subsidiary that BioPontis established to strengthen the financial bridge to cures.

“It is through collaboration with world-class research institutions, such as Massachusetts General Hospital and the Flemish Institute for Biotechnology (VIB) that we can reach our goal of bringing new and exciting basic research forward to the biopharmaceutical industry for clinical trials, manufacturing and distribution to rare disease patients.”, said Richard A. Basile CEO and Co-Founder at BioPontis Alliance.

Rare diseases taken together represent one of the largest underserved patient populations worldwide, more than twice the HIV/AIDS patients and cancer patients *combined*. The genetic cause of 4000 of the 7-8000 rare diseases are known, but turning that knowledge into therapies remains disorganized and vastly underfunded. This results in only 6% of rare disease patients having an approved therapy today.

#### *About BioPontis Alliance for Rare Diseases*

BioPontis Alliance for Rare Diseases is a 501(c)(3) Public Charity in North Carolina (USA) and a Foundation of Public Utility in Brussels (Belgium) managed jointly by an international Board and executive team. BioPontis Alliance partners with patients’ organizations and academic researchers to bridge the gap from promising science to medicines, ready for uptake by the biopharmaceutical industry. BioPontis Alliance is reaching out to all stakeholders in the biopharmaceutical industry, to institutions, charitable funds and foundations, and to individuals who want to directly impact the healthcare outcomes for rare disease patients who live with no hope of treatment today. Contributions to fund and support our programs and mission can be made at <http://biopontisalliance.org> or by contacting the contact persons below.

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