Biogen Idec, Fondazione Telethon and Ospedale San Raffaele Announce Global Collaboration to Develop Gene Therapies for Hemophilia

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- Programs to apply lentiviral vector technology to hemophilia A and B -

CAMBRIDGE, Mass. & MILAN--(BUSINESS WIRE)--Biogen Idec (NASDAQ:BIIB), Fondazione Telethon and Ospedale San Raffaele have entered into a worldwide collaboration to jointly develop gene therapies for the treatment of both hemophilia A and B. The agreement will combine **San Raffaele - Telethon Institute for Gene Therapy's (TIGET)** extensive expertise in creating new gene therapy strategies and developing them from the bench to bedside with Biogen Idec's deep understanding of hematology to potentially treat the underlying causes of hemophilia A and B.

Under the terms of the agreement, TIGET will receive an upfront payment of $5 million for the rights to the programs and their development, and is eligible to receive further payments subject to the achievement of certain development milestones. In addition, Biogen Idec will support the research costs for the two programs; one in hemophilia A and one in hemophilia B. Biogen Idec will have the option to exercise worldwide development and commercialization rights for each program following the initial clinical proof of concept trials.

"People with hemophilia often require life-long treatment to control dangerous bleeding, but there is hope that gene therapy could one day lead to a single-dose, lasting therapy," said **Olivier Danos**, Ph.D., Biogen Idec's senior vice president of gene therapy. "Collaborating with the gene therapy pioneers at TIGET is an exciting step as we build a world-class platform in an effort to identify new approaches that could fundamentally change the lives of people with life-altering diseases."

The collaboration centers on TIGET's advanced lentiviral gene transfer technology. Lentiviral vectors are engineered viruses used to deliver working versions of defective genes responsible for causing certain diseases into the patients' cells. This approach has shown promise in clinical trials for the treatment of some immune-hematologic and neurodegenerative diseases by gene transfer into hematopoietic stem cells harvested from the patients, treated with the vector and then re-infused into the body. In the TIGET strategy for treating hemophilia, the lentiviral vector is directly administered into the body and targets liver cells. If proved safe and effective, this approach may in the future provide long-term, stable therapeutic benefit in people affected by hemophilia.

"We at TIGET have worked for several years to develop a new vector design that upon administration into the blood stringently targets expression of its genetic cargo to the hepatocytes, the main cell type of the liver. This was crucial to establish long-term expression of the therapeutic gene and obtain proof-of-principle of its therapeutic benefit in experimental models of hemophilia B. We are now delighted to collaborate with Biogen Idec to expand our hemophilia gene therapy program also to hemophilia A and to advance both programs towards clinical testing, always keeping in mind the safety and benefit of the patients," said **Luigi Naldini**, director of TIGET.

"This alliance confirms again how the excellent scientific research we are funding can lead to exciting new therapeutic approaches," said the general manager of Telethon, **Francesca Pasinelli**. "We can say that we have pioneered a model whereby the charity organization acts not only as a funding agency, but plays a primary role in managing the development of research to ensure that each step of the process leads to the ultimate goal, which is to provide accessible therapy to patients."

Hemophilia is a rare, chronic, inherited disorder in which the ability of a person's blood to clot is impaired, due to a mutation in the genes required to induce this process. As a result, people with hemophilia experience prolonged bleeding episodes that may cause pain, irreversible joint damage and life-threatening hemorrhages. The aim of gene therapy in hemophilia is to create functioning genes that allow patients to produce clotting proteins on their own. If proven effective and stable, gene therapy may one day provide a single-dose, lasting therapy for hemophilia.

About Hemophilia A and B
Hemophilia A occurs in about one in 5,000 male births annually, and more rarely in females, affecting about 16,000 people in the United States. Hemophilia B occurs in about one in 25,000 male births annually, and more rarely in females, affecting about 3,300 people in the United States. The World Federation of Hemophilia global survey conducted in 2012 estimates that approximately 142,000 people worldwide are identified as living with hemophilia A and approximately 28,000 people are currently diagnosed with hemophilia B worldwide.

About Biogen Idec
Through cutting-edge science and medicine, Biogen Idec discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For product labeling, press releases and additional information about the Company, please visit http://www.biogenidec.com.

About San Raffaele -Telethon Institute for Gene Therapy (TIGET)
Settled in Milan, Italy, the San Raffaele-Telethon Institute for Gene Therapy (TIGET) is a joint venture between the San Raffaele Hospital and Scientific Institute and the Telethon Foundation established in 1995 to perform research on gene transfer and cell transplantation and translate its results into clinical applications of gene and cell therapies for different genetic diseases. For more information, please visit the Institute website, http://www.tiget.it/. San Raffaele Hospital is part of San Donato Hospital Group since 2012.

Safe Harbor
This press release contains forward-looking statements, including statements about Biogen Idec's expectations and mission to develop new therapies for people with hemophilia, through its collaboration with **San Raffaele -Telethon Institute for Gene Therapy (TIGET).** These forward-looking statements may be accompanied by such words as "anticipate," "believe," "estimate," "expect," "forecast," "intend," "may," "plan," "will" and other words and terms of similar meaning. You should not place undue reliance on these statements. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including risks and uncertainties associated with drug development and commercialization, Biogen Idec's dependence on third parties over which it may not always have full control, and the other risks and uncertainties that are described in the Risk Factors section of Biogen Idec's most recent annual or quarterly report filed with the Securities and Exchange

Commission. These statements are based on current beliefs and expectations and speak only as of the date of this press release. Biogen Idec does not undertake any obligation to publicly update any forward-looking statements.

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