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Bringing Hope to XLHED

Edimer Pharmaceuticals Presents Update on Phase 2 Clinical Trial of EDI200 at International Gathering of Ectodermal Dysplasia Patient Foundations

-- Company seeks collective effort from global community to support completion of accrual of first potential treatment for rare genetic disorder XLHED—

Cambridge, Mass. – April 01, 2014 – Edimer Pharmaceuticals, a biotechnology company focused on developing an innovative therapy for the rare genetic disorder, X-linked Hypohidrotic Ectodermal Dysplasia (XLHED), today announced that Neil Kirby, Ph.D., President and Chief Executive Officer of Edimer, presented an update on the company's lead compound, EDI200, to the International Ectodermal Dysplasia Network meeting in Milan, Italy. This tri-annual meeting is designed to promote international collaboration and share experiences related to the management of ectodermal dysplasias including XLHED, which is a serious and potentially life-threatening disorder associated with hyperthermia and clinically significant respiratory infections in addition to hair and tooth malformations. This was the first time the meeting was convened since a potential treatment entered evaluation in a clinical trial.

The initial phase 2 clinical trial of EDI200 is being conducted at several European and three U.S. medical centers. EDI200 replaces EDA-A1, the protein missing in XLHED and a key regulator of skin and tooth development. If fully developed and approved, EDI200 will be the first protein therapeutic to provide a sustained correction of the symptoms of this disorder.

"The development of EDI200 is a very promising advance that has the potential to positively impact the lives of children born with XLHED by offering them a clinically-significant, life-long health benefit," said Kathy Grange, MD, professor of pediatrics at Washington University School of Medicine in St. Louis. "Women that know or suspect that they are carriers of XLHED and who are pregnant or thinking of becoming pregnant may have an opportunity for their newborn son to participate in the EDI200 clinical trial."

"Our mission since our founding 33 years ago has been to empower and connect people touched by ectodermal dysplasias through education, support, and research. This is the first time in our history that we might also help provide our families hope for a potential therapy," said Mary Fete, M.S.N., R.N., C.C.M., Executive Director of the National Foundation for Ectodermal Dysplasias (NFED). "We are grateful for Edimer's continued involvement in the International Ectodermal Dysplasia Meeting, their generosity in offering an unrestricted grant to cover the travel costs of many of the numerous meeting participants and their passionate pursuit of a valuable treatment option for families with XLHED."

"The international XLHED community has been an invaluable partner in Edimer's effort to develop a treatment for this serious, potentially life-threatening disorder. We have shared scientific and medical knowledge among the patient advocacy community worldwide and collaborated on the development of an international patient registry," said Neil Kirby, Ph.D. "With the EDI200 neonate phase 2 trial

underway, we look to our partners in the global XLHED community to share information about this clinical trial and help us identify patients who are eligible to participate."

Dr. Kirby's presentation provided an update on progress to date on the phase 2 clinical trial as well as an overview of outreach efforts to identify and recruit additional patients. Dr. Kirby also lead a discussion among meeting participants regarding ways the foundations can work together, and in collaboration with Edimer, in advancing this investigational therapy.

About the Phase 2 Clinical Trial

The phase 2 clinical trial is designed to evaluate the safety, pharmacokinetics, pharmacodynamics and efficacy of EDI200 in XLHED-affected male newborns in the first two weeks of life. EDI200 dosing will be initiated between the 2nd and 14th days of life, with each study subject receiving two doses per week for a total of five doses. For additional information on this clinical trial, please visit clinicaltrials.gov, identifier NCT01775462.

About EDI200

EDI200 is an ectodysplasin-A1 (EDA-A1) replacement protein, representing the first of a new class of molecules rationally designed to correct a specific developmental disorder. EDI200 has been shown to bind specifically to the EDA-A1 receptor (EDAR), activating the signaling pathways that lead to normal ectoderm development. EDI200 has demonstrated substantial and durable efficacy in animal models of XLHED with notable reduction in mortality and morbidity. The U.S. Food and Drug Administration (FDA) granted Orphan Drug designation and Fast Track status to EDI200. EDI200 also has Orphan Drug designation in Europe.

About XLHED

XLHED (also known as Christ-Siemens-Touraine Syndrome) is a rare disorder of development resulting from genetic mutations in the ectodysplasin gene (EDA). Patients affected by XLHED are at risk for life-threatening hyperthermia based on their inability to regulate body temperature, and for clinically-significant pneumonias resulting from their abnormality in respiratory secretions. Cardinal signs and symptoms in XLHED include diminished/absent sweat, reduced and abnormal airway secretions, few and often misshapen teeth, and absent or early hair loss from face and scalp.

XLHED patients surviving infancy are predisposed to atopy presenting with eczema and asthma, chronic sinusitis, recurrent nose bleeds, and dry eye complications. Almost uniformly they require dental interventions including early prostheses and later implants. Their susceptibility to hyperthermia, may impact normal participation in outdoor activities, sports and school attendance. Both medical and self-esteem issues are life-long in this disorder. As is generally true with X-linked inheritance, males are fully affected while females are variably affected.

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About Edimer Pharmaceuticals

Edimer is a privately held biotechnology company based in Cambridge, Massachusetts dedicated to delivering a significant and durable improvement in the health and quality of life for future generations affected by XLHED. Edimer was established in 2009 with investment from Third Rock Ventures and VI Partners. NEA and Sanofi-Genzyme BioVentures joined the initial investors in a Series B round of equity financing that closed in July of 2013.

For further information on Edimer Pharmaceuticals, please visit www.edimerpharma.com. To receive regular updates about Edimer Pharmaceuticals' progress please join the XLHED network at www.xlhednetwork.com. For further information on Edimer Pharmaceuticals, please visit www.edimerpharma.com.

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