Dermelix Biotherapeutics Announces Partnership with EspeRare for Pivotal-stage Antenatal Protein Replacement Therapy for XLHED

- Dermelix has formed a partnership with EspeRare to develop and commercialize DMX-101 for a rare pediatric disease called XLHED

- Dermelix and EspeRare anticipate initiating a single pivotal study in the US and Europe in the second half of 2019

- If approved, DMX-101 has the potential to be the first ever antenatally administered drug

NEW YORK, April 2, 2019 /PRNewswire/ -- **Dermelix Biotherapeutics**, a biopharmaceutical company focused on the development of innovative treatments for rare genetic skin diseases, today publicly announced its partnership with EspeRare, a not-for-profit organization dedicated to accelerating the development of rare diseases treatments, for DMX-101, a novel in utero protein replacement therapy for the rare pediatric disease X-Linked Hypohidrotic Ectodermal Dysplasia (XLHED).

XLHED is caused by a genetic deficiency of ectodysplasin A (EDA), a protein required for the normal development of ectodermal structures before birth including sweat glands, respiratory glands, skin, hair, teeth, and other structures. Major debilitating clinical manifestations of XLHED include life-threatening hyperthermic episodes due to the inability to sweat, heat intolerance, and increased risk of serious respiratory tract infections due to abnormal respiratory gland function, resulting in increased mortality in the early years of life. There are currently no approved therapies for the corrective treatment of XLHED and the current standard of care is supportive only.

DMX-101 is a first-in-class protein therapy designed to replace the function of endogenous EDA during late fetal development through a single-course of treatment delivered into the amniotic fluid during the later stages of pregnancy. This approach has already demonstrated significant potential in humans by normalization of sweat gland function and associated thermoregulation, and improvements in dentition and respiratory function observed in a case series of three patients treated in utero with DMX-101 during the third trimester of pregnancy. These results were recently published in the New England Journal of Medicine (N Engl J Med 2018; 378: 1604-1610) and featured in Nature Medicine's 2018 Research Highlights (Nature Medicine 2018; 24: 702).

Caroline Kant, Founder and CEO of EspeRare commented, "This partnership with Dermelix is a turning point to advance XLHED therapy and to improve the lives of the XLHED patients and their families. It is based on our shared values and commitment to engage the patient community at each step of the development."

Under the terms of the agreement, EspeRare will sponsor the development of DMX-101 in Europe, where it benefits from accelerated development under EMA's PRIME (PRIority MEdicines) scheme and was developed under the name ER-004. Dermelix will sponsor the development of DMX-101 outside of Europe where it benefits from Orphan Drug Designation in the US, and will be responsible for the commercialization of DMX-101 worldwide. Dermelix and EspeRare anticipate initiating a single pivotal study in the US and Europe in up to 15 XLHED patients in the second half of 2019.

Dr. Nick France, Chief Medical Officer of Dermelix commented, "Our partnership with EspeRare represents a major milestone in establishing Dermelix as a leader in the orphan dermatology space, and is emblematic of our vision in accelerating the translation of scientific discoveries into approved therapies for patients with some of the highest unmet medical need."

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About XLHED

XLHED is a severe, chronically debilitating and life-threatening ultra-rare disease affecting approximately 4 per 100,000 live male births per year. XLHED is caused by genetic mutations in the EDA protein, resulting in functional deficiency of this important developmental protein. The absence of functional EDA results in abnormal development of the skin, sweat glands, sebaceous glands, hair, oral cavity, and respiratory mucosal glands resulting in serious life-threatening clinical manifestations from birth including hyperthermia, craniofacial anomalies, recurrent respiratory infections, and concomitant psychosocial comorbidities that impair quality of life in patients and their families.

About DMX-101

DMX-101 is a first-in-class protein replacement therapy comprised of a fully humanized EDA1 molecule consisting of the human IgG1 Fc sequence linked to the human EDA1 TNF binding domain. Preclinically, DMX-101 has been shown to bind to the receptor EDAR resulting in activation of the NFκB signaling pathway, which triggers transcription of genes involved in the normal development of multiple tissue types. DMX-101 has been shown to be a functional replacement of endogenous EDA both preclinically and clinically when administered early in development.

About Dermelix Biotherapeutics

Dermelix is a privately-held, clinical-stage biopharmaceutical company focused on the development of innovative therapies for rare and debilitating dermatologic conditions with high unmet medical need. For more information, please visit http://www.dermelix.com/.

About EspeRare

EspeRare is a Swiss not-for-profit organization that is committed to improve the lives of children with life-threatening rare diseases. EspeRare addresses the unmet medical needs of these children by uncovering the potential of existing treatments. EspeRare's innovative model combines pharmaceutical know-how with philanthropic, public and private investments to develop and bring to life these discontinued therapies. With its unique patient-centered approach to drug development, EspeRare engages the patient community at each step of the process, with the intent of giving children and their families fair access to these therapies and a new hope for the future. For more information, please visit www.esperare.org**.**