



*FOR IMMEDIATE RELEASE*

*Corporate press release*

## **Promethera Announces Review Acceptance of Heparesc New Drug Submission for Neonatal Onset Urea Cycle Disorders by Health Canada**

**Mont-Saint-Guibert, Belgium, July 3, 2017** – Promethera Biosciences SA, a global innovator in cell-based medicines, today announced that Health Canada has accepted its New Drug Submission (NDS) of Heparesc in neonatal onset Urea Cycle Disorders (UCD) for review. UCDs are ultra-rare genetic disorders causing the accumulation of toxic ammonia levels for which liver transplantation is currently the only curative, but not immediately available, treatment to correct the metabolic defects. Heparesc is composed of cryopreserved mature human liver cells (hepatocytes) obtained from ethically donated livers. The fully functional mature human hepatocytes aim to substitute missing deficient urea cycle functions in neonatal onset patients' livers. Heparesc could serve as a bridge to transplantation while increasing metabolic stability.

"This achievement is a recognition of our dedication to working with the Canadian regulatory bodies as a start to build a path for innovative cell therapies for patients worldwide and a validation of our approach for severe liver diseases," said John Tchelingierian, CEO of Promethera. "While our strategic focus remains on our stem/progenitor cell products such as Hepastem and H2Stem and the larger indications ACLF and NASH, I am very proud of this joint effort which is a prime example of the successful integration of the Cytonet and Promethera teams."

The acceptance of the NDS completes the validation period and marks the start of a 300-day review period by Health Canada. Upon potential successful outcome of the review, Promethera would be able to commercialize Heparesc in neonatal onset UCD in Canada. The New Drug Submission is based on two successfully completed studies of Heparesc, which is one of the assets Promethera gained access to through the acquisition of Cytonet in April 2016.

### **About Urea Cycle Disorders**

Urea Cycle disorders (UCD) are severe genetic disorders caused by mutations in the enzymes of the urea cycle. These enzymes participate in the removal of nitrogen from the body, converting it into urea, which is excreted via the urine. In case of enzyme deficiency nitrogen accumulates in the form of the toxic substance ammonia which can cause irreversible brain damage, coma and even death. The severity of the disease and the age-of-onset depend on the affected enzyme and the severity of the mutation.

### **About Heparesc**

Heparesc is composed of cryopreserved mature liver cells (Human heterologous liver cells (HHLivC)), obtained after disaggregation of donated livers, which can be easily reconstituted at the patient treatment site and infused into the portal vein of the recipient liver.

### **About Promethera Biosciences**

Promethera Biosciences is a global innovator in liver cell-based medicines whose mission is to help patients overcome acute and chronic liver diseases. Our lead clinical program, derived from our patented



cell technology platform HepaStem, is designed to benefit from its immune-modulatory and anti-fibrotic properties. We are a team of international experts operating out of R&D and GMP facilities in Mont-Saint-Guibert, Belgium, and Durham, NC, USA.

Promethera®, HepaStem®, H2stem®, Cytonet®, Heparesc® are all registered trademarks of the PROMETHERA group.

**Contact Information:**

**Promethera Biosciences SA**

Alexandra Schiettekatte

[Alexandra.schiettekatte@promethera.com](mailto:Alexandra.schiettekatte@promethera.com)

Web: [www.promethera.com](http://www.promethera.com)

**For media:**

MacDougall Communications

Gretchen Schweitzer

or Mario Brkulj

+49 89 2424 3494

or +49 175 501 0575

[mbrkulj@macbiocom.com](mailto:mbrkulj@macbiocom.com)



<https://www.linkedin.com/company/promethera-biosciences>



<https://twitter.com/Promethera>