



## **MITHRA RECEIVES ORPHAN DRUG DESIGNATION FROM EMA FOR E4 IN NEONATAL ENCEPHALOPATHY**

- **Orphan Drug Designation received from European Medicines Agency for Estetrol (E4) in the treatment of life-threatening Neonatal Encephalopathy**
- **Evidence of potential of E4 to address additional indications beyond Women's Health**
- **Preclinical results form basis for potential additional partnerships for E4**

**Liège, Belgium 9 June 2017** – Mithra (Euronext Brussels: MITRA), a company dedicated to Women's Health, today announces that it has received Orphan Drug Designation (ODD) for E4 in Neonatal Encephalopathy (NE). Mithra intends to develop E4 to treat Hypoxic Ischemic Encephalopathy (HIE), a subset of NE which accounts for 50-80% of cases and affects pre-term and new born babies (>36 weeks gestation)<sup>1</sup>.

Based on promising preclinical results of E4 in HIE, including improvements in pathophysiology, general well-being and motor function, the EMA granted ODD Designation for E4 in this indication. Although Mithra's primary focus is on Women's Health, the Company is investigating E4's potential in a wider range of indications including neuroprotection and wound-healing. The preclinical study in HIE was conducted in collaboration with the University of Liège, Belgium. Mithra intends to advance the study of the potential neuroprotective properties of E4 in HIE over the next two years and seek a partner for further clinical development.

HIE is a condition affecting approximately 30,000 newborns each year in the European Union and the U.S.<sup>2</sup> HIE is a consequence of the reduction in the supply of blood or oxygen to the baby's brain before, during or shortly after birth. With approximately 25% of infants dying prior to discharge from the neonatal intensive care unit, HIE is a major cause of mortality. Moreover, research shows severe neurological impairment and long-term disability among survivors, with 46% affected at 18-22 months' follow-up<sup>3</sup>. Currently, HIE is treated with therapeutic hypothermia, or 'cooling' the infant in

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<sup>1</sup> Kurinczuk et al. *Early Hum Dev* 2010; 86: 329-338, 2010.

<sup>2</sup> Kurinczuk et al. *Early Hum Dev* 2010; 86: 329-338, 2010.

<sup>3</sup> Shankaran et al. *Early Hum. Dev.* 1991; 25(2):135-148.

order to reduce brain damage, but this treatment has limited efficacy and comes at a high cost<sup>4,5</sup>. Hence, HIE remains a serious unmet medical need.

**François Fornieri, CEO of Mithra Pharmaceuticals, commented:** *"The ODD for E4 in Neonatal Encephalopathy underlines the potential of our unique natural E4 estrogen platform in areas beyond Women's Health, including neuroprotection. HIE is a serious and prevalent syndrome under the umbrella of NE that causes significant mortality and morbidity in infants.*

*Limitations with current treatment options in terms of efficacy and access highlight the unmet medical need. Given the promising initial preclinical data and the ODD, we look forward to exploring this important indication further."*

## Pictures

For pictures of François Fornieri, please click here on the following link:  
<http://www.mithra.com/en/press-media>

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## About Mithra

Mithra (Euronext: MITRA) is dedicated to providing innovation and choice in women's health, with a particular focus on fertility, contraception and menopause. Mithra's goal is to develop new and

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<sup>4</sup> Cotten & Shakarn. *Expert Rev Obstet Gynecol*. 2010; 1;5(2):227-239.

<sup>5</sup> Regier et al. *Value Health*. 2010; 13(6):695–702.

improved products that meet women's needs for better safety and convenience. Its two lead development candidates - a fifth generation oral contraceptive, Estelle®, and a next generation hormone therapy, Donesta®- are built on Mithra's unique natural estrogen platform, E4 (Estetrol). Mithra also develops, manufactures and markets complex therapeutics and offers partners a complete spectrum of research, development and specialist manufacturing at its Mithra CDMO. Mithra was founded in 1999 as a spin-off of the University of Liège by Mr. François Fornieri and Prof. Dr. Jean-Michel Foidart. Mithra has an approximate headcount of 140 staff members and is headquartered in Liège, Belgium. Further information can be found at: [www.mithra.com](http://www.mithra.com)

### **Important information**

*The contents of this announcement include statements that are, or may be deemed to be, "forward-looking statements". These forward-looking statements can be identified by the use of forward-looking terminology, including the words "believes", "estimates," "anticipates", "expects", "intends", "may", "will", "plans", "continue", "ongoing", "potential", "predict", "project", "target", "seek" or "should", and include statements the Company makes concerning the intended results of its strategy. By their nature, forward-looking statements involve risks and uncertainties and readers are cautioned that any such forward-looking statements are not guarantees of future performance. The Company's actual results may differ materially from those predicted by the forward-looking statements. The Company undertakes no obligation to publicly update or revise forward-looking statements, except as may be required by law.*

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