c4c Multi-Stakeholder Meeting on Perinatal Asphyxia

18-19 September 2023
Ergife Palace Hotel, Rome
& Zoom

Call for Expression of Interest

conect4children is pleased to announce that the fourth c4c International Multi-Stakeholder Meeting (MSM) will address **the topic perinatal asphyxia.** The meeting will be a **hybrid** meeting that will take place **on 18 and 19 September 2023, ahead of the 5th jENS (joint European Neonatal Societies) conference in Rome.**

Multi-Stakeholder Meetings *have been conceived with the aim to* ***facilitate dialogue and provide an opportunity for constructive interactions between relevant stakeholders*** *(patients/patient representatives, clinicians, academics, pharmaceutical companies and regulators from EMA and FDA)* ***on topics requiring open discussion on development of medicines in the best interests of neonates, children and adolescents.*** *The goal of these meetings is to share information, in a pre-competitive setting, to define unmet medical needs, to define how best to address those needs and facilitate the development of innovative medicines towards eventually their introduction into the standard-of-care of neonates, children and adolescents.*

Rationale

Despite advances in perinatal medicine, the incidence of perinatal asphyxia (PA) has not decreased in the last decade. **Perinatal asphyxia occurs in 5.4/1000 live born neonates, with 1.8/1000 live births diagnosed with hypoxic ischemic encephalopathy (HIE).** It is an orphan disease, as the Committee for Orphan Medicinal Products estimates that about 15 000 neonates (0.3/10 000 EU inhabitants) are affected annually. Intensive care unit admission with multiple drug treatment is often needed. Whole body cooling therapy, also known as therapeutic hypothermia, is hereby used as standard of care in neonates with PA and HIE since 2010. **Cooling [i.e. core body temperature of 33.5°C for 72 h] reduces mortality and neurodevelopmental disability in term and late preterm neonates with moderate-to-severe HIE** if initiated before 6h of age, with a number needed to treat of 7 to result in one additional survivor without neurological impairment. Consequently, the benefits of cooling on survival and neurodevelopment clearly outweigh the short-term adverse effects. These benefits are due to reduced metabolic rate, decreased neuronal apoptosis and/or modulation of the inflammatory response. However, PA still accounts for a relevant proportion of neonatal mortality and morbidity in (near)term neonates, also in cooled cases (45 %), so that **interventions to further improve outcome are urgently needed in addition to cooling**.

The specific setting to conduct clinical trials in this patient group (taking into account the orphan disease, diagnosed very shortly after birth, unanticipated and no clustered care at diagnosis, the urgent consent issues, uncertainties on relevant biomarkers for severity and outcome, uncertainties on how to model

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389.
The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.

target exposure, the challenges and limitations of the 'commonly used' randomized-placebo controlled trials).

Specific objectives

- Update on science;
- Discuss and define preclinical evaluation and a preclinical path to a reach a consensus and provide a guideline for both the academic community and pharma development;
- To reach a consensus on which biomarkers to be used and consider a qualification process;
- To address the hurdles in feasibility of clinical development including maximising patient recruitment and propose solutions.

The overarching objective of the meeting is **to propose a strategy to improve the timely development and access to additional therapies to therapeutic hypothermia**, properly addressing paediatric unmet needs, introducing innovative development pathways in the regulatory environment and increasing accessibility for all patients.

Outputs

The outputs of the meeting will be:

- an article in a peer reviewed
- guidelines to be submitted to authorities for qualification/endorsement
- a research proposal for a multistakeholder public private initiative.

No regulatory decisions will be made during the meeting, which will focus on publicly available information. However, the findings and conclusions of the meeting will pave the way for future development plans and concrete actions.

Who can participate?

International academic experts in perinatal asphyxia, experts in neonatal preclinical and biomarker research, representatives from pharmaceutical companies developing relevant assets, representatives from regulators (EMA, FDA) and patients, parents and their advocates.

The meeting will be **on invitation only** following expression of interest. Interested participants can apply by filling the **Expression of Interest form** available here below

Submit your Expression of interest

If the button does not work, please use this LINK

Deadline for submission: **30 July EOB (Brussels time)**

Invited participants will be contacted in due time.

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389.
The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.