

RDR Challenge

Delivery system for intranasal administration of biological drugs to neonates

INDUSTRY SPONSOR

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AIM

To develop a delivery system allowing administration of liquids or gels in nostrils of the neonates for intranasal administration of biological drugs.

BACKGROUND AND RATIONALE

One of the biggest challenges in the rare disease field is the efficient delivery of therapeutic agents into the central nervous system (CNS) to target neurological symptoms. Currently, CNS drug delivery is achieved through invasive routes that bypass the blood brain barrier, such as intrathecal, intracerebroventricular or intraparenchymal injections that deliver directly to the cerebrospinal fluid (CSF) of the CNS. Although being currently employed in the clinical setting, the use of such techniques is limited as the risk of infections is very high. A promising strategy to bypass the blood-brain barrier is the delivery of drugs from the nose to the brain via Intranasal (IN) route. This is recognized as one of the most useful and reliable routes for brain drug absorption leading to quick drug action. In addition, intranasal administration bypasses gastrointestinal and hepatic metabolisms, thus enhancing drug bioavailability. Devices for IN delivery of liquid or lipid-based particulate formulations are already available in the market, however systems able to efficiently deliver biological drug formulations (e.g. viscous/semisolid cells suspensions, solutions of antibodies or large proteins) in the brain through nasal cavities are still lacking.

BENEFITS FOR RARE DISEASES

There are many rare conditions of the neonates (both term and preterm) that affect the CNS and require babies to be medically assisted with drugs delivered in the brain with the most efficient and delicate techniques. The development of a IN-delivery system for biological drugs specific for CNS targeting in the neonates would allow a safe and efficient administration of biological drugs minimizing product loss while increasing drug availability.

HORIZON SCANNING

A preliminary analysis of the area has been performed and to date, to the best of our knowledge at the current stage, there is not such a device (both marketed or under development) with the ability to intranasally deliver biological drugs to neonates. The majority of devices for IN delivery of drugs are either for adults or not applicable to biological drugs. Among companies developing devices for IN delivery we found **Shin Nippon Biomedical Laboratories** <https://www.snbl-nds.com/technology/>. Their technology can be applied to a wide range of drugs, including small molecules, peptides, and biologics. It can be utilized for improved systemic drug absorption, enhanced muco-immunity for a vaccine, or for enabling drug delivery to the brain through the olfactory area. However, the device is for adults only and can't be adapted to neonates needs.

A project which might be of interest for this challenge is the **N2B-patch project** - an EU-funded project that started in January 2017. <https://www.n2b-patch.eu/> or <https://cordis.europa.eu/project/rcn/206572/factsheet/en>

They declare the following: “We aim to develop an innovative N2B drug delivery technology for the treatment of MS based on the synthesis of a biomaterial-based innovative galenic formulation, a novel class of hydrogel matrix-patch and a medical applicator device system. The N2B route of administration provides a minimally invasive method of bypassing the blood-brain-barrier (BBB). Moreover, the innovative multifunctional galenic formulation of N2B-patch aims to shield sensitive drugs and maintain the drug’s bioactivity.”

The device is actually not developed for delivery to neonates, but might be adaptable. Further contact with the company is necessary to better understand if they could be interested to apply to the call.

TIMELINES/MILESTONES AND DELIVERABLES

Stage 1 (M18):

- Development of the prototype
- Intermediate step: engagement with EMA to validate the technical profile through ITF (early interactions on innovation)

Stage 2 (M30):

- Demonstration of efficient delivery of different biological drug formulations in the brain in vivo in large animals (most probably non-human primates, NHP). This stage will include the treatment of at least 2 animals and tissue analysis (12 month-period).
- Obtain CE marking at the end of second stage is optional (preferred).

EXPECTED CONTRIBUTION AND EXPERTISE

Expertise in biomedical devices, biologics drug product formulation is preferred (especially if developed in neonatology therapeutic area). Optimal IN devices should not harm the nasal mucosal surface while ensuring reproducibility of drug administration and avoiding drug loss in the nasal mucus/cavity.

TOTAL BUDGET: 487.500 €

Contribution from the sponsor

In kind:

- Consolidated experience in Neonatology and biotech product formulation.
- Chiesi can also provide some biological material to be used for testing the device.

Financial:

Project Name	Total budget (euros)	N° of industrial partners	Min % cash contribution from industrial partner	Cash contribution from industrial partner included in total budget
IN Device project	487 500	1	30%	112 500 (30%)