CREAPHARM CLINICAL SUPPLIES

CREAPHARM BIOSERVICES

NOVEMBER 2022

UNPARALLELED SUPPORT

STUDY CASE#1

ot4b BRIGHTEN LIFE PATH

Managing the set-up and clinical supply operations in the OTBB3 phase III clinical trial

EXTENDED SUPPORT TO SUCCESSFULLY ACHIEVE A QUALITY RAPID START-UP WITHIN A TIGHT TIMELINE

OT4B is a French start-up, resulting from an alliance between academic research and a patient association, with the **aim of providing the first early treatment for Prader-Willi syndrome (PWS)**.
The objective of this oxytocin treatment is to modify the disease trajectory for patients suffering from this rare condition, by intervening from the first weeks of life.

THE CLINICAL TRIAL

European phase III OTBB3
Neonates and Infants With Prader-Willi Syndrome (PWS)

Started in March 2020, OTBB3 is a prospective, randomized, placebo-controlled, phase III trial to assess the safety and efficacy of 4 weeks oxytocin administration on oral and social skills in neonates/infants with PWS.

In 2018, **OT4B started looking for a partner specialist in clinical supplies, able to fully meet its needs**, including:
- pick-up of Drug Product at the manufacturer site (France)
- clinical secondary packaging and clinical labelling of patient kits
- QP-release
- European clinical distribution management to investigational sites
- kit assembly and biospecimen management (material sourcing, pick-up on sites, intermediate storage & shipment to central/specialized laboratories)

> **We benefited from a proactive and responsive QP support which enabled us to start the clinical study on time**

Anne-Laure Astecker
Director of Regulatory and Pharmaceutical Affairs, OT4B

#PARTNERSHIP

Our extra wish: partnering with a French company. When we visited CREAPHARM for the first time, we really enjoyed their team spirit and felt we can build a trust-based relationship.
Carole Gueroult
Head of Clinical Operations, OT4B

OT4B always shares with the CREAPHARM Project Team their research purposes and what patients do expect.
A driver to work hand in hand, with a patient centric approach.
Delphine Courade
Head of Project Management, CREAPHARM CLINICAL SUPPLIES

WORKING HAND IN HAND TO SPEED UP ACCESS TO TREATMENT FOR PATIENTS

Early anticipation of the clinical supply management

If biotech companies are often focused on drug development and manufacturing challenges, the clinical packaging and logistics of the investigational products need to be prepared and scheduled.



Always offering solutions to face unexpected upstream delays

While OT4B was managing in conjunction manufacturing of its Drug Product and had booked slots for further clinical packaging and labeling operations, any delay related to the first steps had consequences on the timeline.
The CREAPHARM's team agility has benefited to OT4B: each patient in the OTBB3 phase III clinical trial has received its treatment on time. Responsiveness, a real strength.

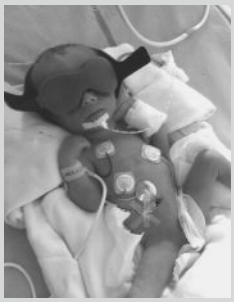
A long-lasting partnership

While managing clinical supplies in OTBB3 Phase III clinical trial initiated the partnership between OT4B and CREAPHARM, several other contracts have been signed since 2019. CREAPHARM is delighted to continue to support OT4B in its different clinical trials, follow-up studies as well as to manage their compassionate use drugs.

About Prader-Willi syndrome (PWS)

A rare genetic disorder, a syndrome which includes severe neonatal hypotonia with impaired sucking and swallowing leading to failure to thrive in the most severe cases, subsequently followed by an early onset of morbid obesity with insatiable hunger, combined with other endocrine dysfunction probably due to hypothalamic dysfunction.

A **deficit in oxytocin has been reported** (1).
There is **currently no therapeutic solution for the treatment of the youngest patients with PWS.**
Growth hormone is the only medication authorised for the indication of PWS.



The hope of a treatment

Early administration of oxytocin could thus have **a major impact on the restoration of feeding abilities but also on the social behaviour** of children with PWS (2). An effect of this type has been shown in animal models.
The phase II trial OTBB2 showed that intranasal administration of oxytocin, normalises sucking and significantly improves feeding problems, social behaviour and mother-infant interactions (3).
The OTBB3 trial aims at confirming the OTBB2 results. The follow-up of these children will also allow to confirm whether early treatment has a positive long-term impact on their neurodevelopmental trajectory. **Oxytocin would thus be positioned as a real 'Disease Modifier'.**
Another ongoing study aiming at demonstrating the effects of oxytocin on dysphagia in children (2-18y) is currently ongoing.

(1) SWAAB, 1995; SCHALLER, 2010; MUSCATELLI, 2000
(2) SCHALLER, 2010; MEZIANE, 2015
(3) TAUBER, 2017; VIAUX-SAVELON, 2016

QUICK FACTS

01

OT4B'S CHALLENGES

Expecting a full-service approach including extensive support
While preparing the OTBB3 Phase III clinical trial, OT4B was a young biotech company, still staffing, when starting partnering with CREAPHARM.

Dealing with Cold-Temperature conditions (2-8°C) with no TOR (Time Out of Refrigeration)

Entrusting the QP-Release to a certifying site

02

CREAPHARM'S APPROACH

IMP waste limitation
Guidance for packaging runs and distribution plan optimization, based on:
- study protocol & predictive duration
- manufacturing scheduling
- product shelf-life
(on going stability studies)

#1challenge1solution
Providing OT4B with tailor-made solutions for both packaging & logistic operations

Deadline commitment to meet the FPI objective

03

CREAPHARM'S SOLUTION

A **dedicated Clinical Supply Manager** supported by specialists

A **packaging designed** to meet the patient need

Several packaging runs

Operations in cold chamber to remain within the specified limits

Strong **pharmaceutical support**
Quality & regulatory advice,
QP-release and shelf-life statement

Rescheduling & **expediated services** in case of manufacturing lags

Efficient & reliable logistic pathway for both IMPs and patient's samples