Case Study | April 2023

# Cerba Research fights Spinal Muscular Atrophy 7 days a week – a multi-centre prospective and validation study

**Cerba Research** – where the medical world meets the world of logistics.

The world seems smaller in this digital era, information can reach you within a second. However, logistics still poses a challenge in our business. Serum or blood samples that have been collected anywhere in the world, must go to a either a partnering laboratory or our facilities in Rotterdam.

And when it comes to treatment for babies, serum samples must be handled with the utmost speed and special care. We mention babies because of a life changing therapy Viroclinics-DDL is taking part in; the fight against Spinal Muscular Atrophy.

## The treatment

### So how do we treat this disease?

One of the treatments is based on gene therapy. This treatment – which is given by a viral vector – is one of the possible treatments for this disease. It contains a replica of the missing gene. The viral vector passes into the motor neurons in the spinal cord and restores function of the missing gene, which then produces proteins necessary for nerve function and controlling muscle movements. It's given via a one-time intravenous infusion which is administered on a patient for over an hour. This single injection helps newborn babies with SMA to develop close to normal and saves their lives. This treatment is being approved in many countries around the world. One of the pivotal steps to evaluate the eligibility of a patient with SMA for the treatment with gene therapy is the existence of antibodies against the viral vector.

## What is SMA?

Spinal Muscular Atrophy or SMA is a rare genetic (inherited) condition caused by a missing or non-functioning (mutated) SMN1 gene. This condition results in rapid and irreversible loss of motor neurons. This then affects main muscle functions like breathing, swallowing and basic movements. Since these neurons control muscle movements throughout the body, a lack of it could cause paralysis, severe muscle weakness, loss of movements and newborns with the most severe type, will without treatment, die at a young age.

## How common is SMA?

SMA is a very rare disease. That's why they call this an orphan disease. 1 in 10,000 newborns have it. About 1 in 54 people carry the genetic defect of SMA and two carriers have a 25% chance of having a child with SMA.

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## Our added value

You can imagine that each serum sample potentially represents a baby's life. Therefore, any logistical challenges must be overcome quickly and smoothly.

There is a lot at stake.
That's where our extensive expertise and knowledge come in.

We have a proven track record in a wide variety of services varying from serum samples collections from over 50 countries, quick delivery to our Rotterdam site for immediate laboratory testing and which will tell if antibodies are an eventually contra-indication for gene therapy, logistics of sampling kits to the hospitals and laboratories. In this project we work with multi-disciplinary teams from Cerba Research and our departments of Clinical Virology. Services and R&D in Rotterdam.

Daily, these teams work closely together to bring in the patient samples, have them tested, check and report the results immediately, 7 days a week. The next steps for the project, considering that the treatment is being approved in more countries and new markets, keep on opening, will be cooperation with external laboratories.

This will allow Cerba Research to centralize and simplify sampling and logistical processes for the regions where difficulties were being faced previously, therefore decreasing transit times for the samples making the treatment available faster.

The goal and ultimate challenge is to minimize the time from sample collection to reporting results to the physician. SARS-Cov-2 still has a huge influence on the process, regarding closed borders and other major restrictions. Unfortunately, wars and conflicts are also to be mentioned. Sometimes challenges already begin in the very first stage, like finding dry ice for transportation. It is very impactful to work on this project. Because we are of course aware that every sample is a baby's life; a life that we might be able to save. Sometimes a delay of one day may already be too late. So, pace and efficiency are utterly important. It's amazing to see the achievements, how this project is growing and developing.

All these achievements you not only experience with your head, but also with your heart.

Want to know more? Visit our website cerbaresearch.com

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