RegenMed

ONE SHEET

PHYSICIANS-OWNED CIRCLES FOR SPINAL MUSCULAR ATROPHY (SMA)

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BACKGROUND

Spinal muscular atrophy (SMA) is a rare genetic neuromuscular disease that progressively weakens muscles, impacting essential functions like breathing, eating, and walking. It is a leading genetic cause of infant mortality. SMA arises from a deficiency in the survival motor neuron (SMN) protein, crucial for motor neuron function.
The severity of SMA varies widely, categorized into types based on age of onset and motor milestones achieved. Historically, individuals with the most severe forms faced a very limited lifespan.

For many years, SMA lacked effective treatments, leaving families with few options. However, significant advancements in genetic therapies have emerged in recent years, transforming the landscape for individuals with SMA. These breakthroughs highlight the critical role of ongoing research and development in addressing rare diseases and improving patient outcomes. While these new therapies offer hope, challenges remain in areas such as newborn screening for early diagnosis, ensuring equitable access to treatment, and understanding the long-term effects of these novel interventions.

POCS FOR SMA

POCs are by definition the sustained collaboration among physicians around the world on an indication- and treatment-specific clinical study.

Each Circle study is defined by an Observational Protocol (OP), in turn representing a single anatomical region, pathology, treatment protocol and longitudinal outcomes assessment.

A POC thus generates from primary sources (physicians and patients) clinically and statistically significant datasets. All POC datasets are HIPAA, GDPR and Part 11 compliant.

Our Spinal Muscular Atrophy (SMA) Circle is designed to collect clinical data to improve patient outcomes. Please see more here.

An overview of POCs is here. A description of the physician and patient user experiences is here.

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Benefits For Clinicians

For practitioners treating or otherwise interested in a specific orphan disease, POCs offer the following advantages:

- Sustained collaboration with peers and experts in developing clinically-significant longitudinal datasets.
- Minimal cost and burden.
- Earlier, more accurate diagnoses.
- Evidence-based protocols.
- Deeper patient engagement, education.
- 85% of resulting license fees to POC Members.
- Professional advancement (articles, conference presentations, etc.)

Benefits For Industry

For product manufacturers, clinical researchers, and value-based organizations, POCs offer several benefits:

- Validatable, clinically-relevant datasets at a fraction of typical costs.
- Integrated and longitudinal support for regulatory submissions.
- No gaps, errors, undisclosed data manipulations, ownership claims, clinical irrelevance and other weaknesses inherent in "big data" solutions.
- Enable adaptive trial designs and single-arm studies, without compromising rigor.
- Long-term outcomes directly correlated to original clinical hypotheses.
- Ability of Sponsor to define Observational Protocol.
- Multi-center studies enlarging patient populations.
- Access to global experts and current clinical/scientific hypotheses.
- Continued post-market surveillance.

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