Utilization of Real-World Observational Data to Study the Safety and Effectiveness of Spinal Muscular Atrophy Treatments

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Conclusions

- **Biogen supports SMA disease registries across the globe to meet the needs of the SMA community, health-care providers, researchers, regulators, and payers.**
- **Biogen's efforts are aimed at bolstering the capabilities of SMA disease registry efforts to fulfill research needs within Biogen and the SMA community, and at supporting post-marketing requirements and research.**

Introduction

Real-world data are essential to provide robust, timely, real-world safety and effectiveness data for any new therapy in the post-approval setting. This approach has become more relevant since 2015 with the inception of the European Medicines Agency's (EMA) Patient Registry Initiative. As such Biogen has broadly supported a disease registry approach for spinal muscular atrophy (SMA) with multiple partners that can have independent, industry, and regulatory focused objectives. These objectives can include support for the filing, launch, and post-approval strategy, and answer questions including, but not limited to, understanding the natural history of SMA and exploring treatment patterns as well as treatment value.

Objectives

- To establish a global, collaborative network of SMA patient registries.
- To characterize the natural history of SMA.
- To characterize SMA treatment patterns and outcomes. This includes characterizing nusinersen safety and effectiveness in the real-world setting for research purposes, as it is the first approved therapy for SMA.
- To support an ongoing benefit–risk assessment of nusinersen, and fulfill post-authorization commitments and payers' data needs.
- To provide a platform and data to support quality of care assessment and independent development of treatment guidelines.

Methods

- On the basis of guidance from the EMA, Biogen collaborates with SMA disease registries across the globe to gather robust information on SMA disease outcomes to characterize the natural course of SMA and to increase the understanding of nusinersen and other emerging treatments in a real-world setting.
- Over the past 4 years Biogen has worked with registry partners to:
  - Optimize and improve existing data.
  - Align and standardize data on outcomes and safety collected across registries.
  - Support technological tools.
  - Provide financial support to implement data collection.
- The data collected includes data on demographics, clinical characteristics, medical history, functional outcomes, treatments for SMA, and safety data.

Results

- Biogen collaborates with multiple global SMA registries:
  - The International SMA Consortium Registry (ISMAR).
  - Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases (TREAT-NMD).
  - The Muscular Dystrophy Association (MDA).
  - The Canadian Neuromuscular Disease Registry (CNDR).
  - The German SMARTCARE registry.
  - The Polish SMA registry.
  - The Greek SMA registry.
  - The Spanish CUIDAME registry.
  - Dr Haberlova and the READY HCP NMD Registry (Czech/Slovak) investigators.
  - Dr Klein and the Swiss registry for neuromuscular disorders (NMDs) investigators.
  - Prof Fattal and the Israeli SMA registry investigators.
  - The South Korean SMA registry investigators.
- The project involves improving the capacity and capability of the registry partners to allow the obtaining of Global Data Protection Regulation- (GDPR) compliant patient-level data, according to the FAIR (findable, accessible, interoperable, reusable) Principles, to meet the data needs of all stakeholders.
- The resulting medical evidence may have application in:
  - Medical practice (quality of care/guidelines).
  - Regional or local health technology assessment.
  - Regulatory submissions.

Figure 1. Existing and planned SMA registry collaborations.

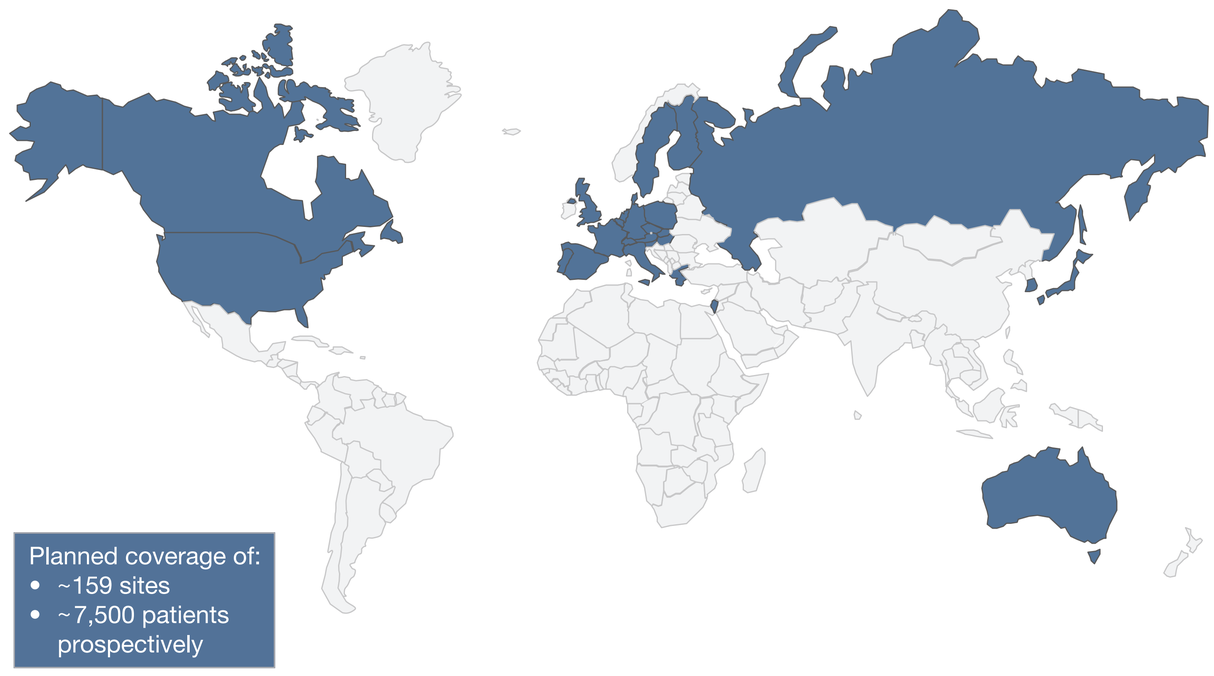
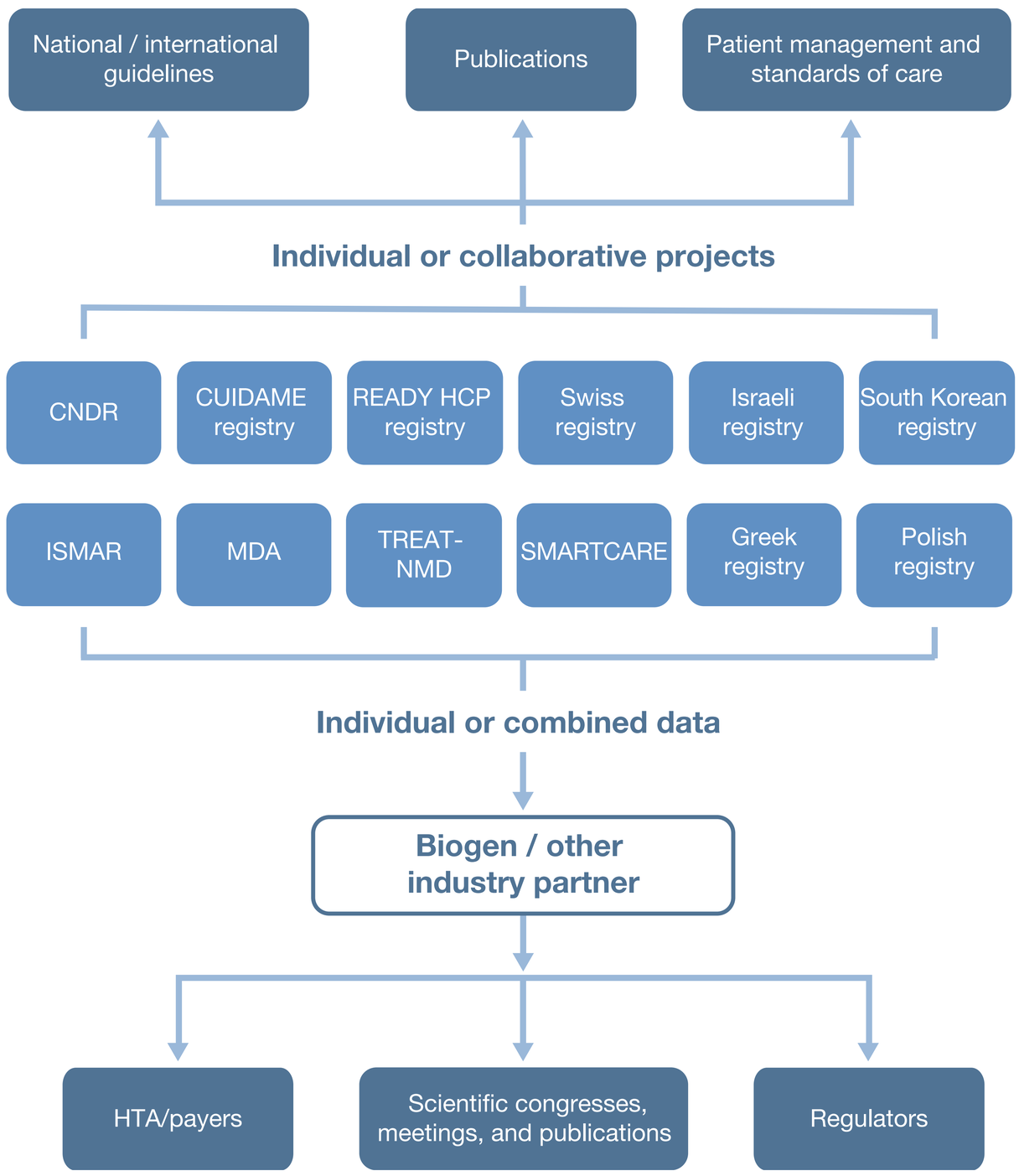


Figure 2. Biogen collaborates with several global registries of SMA patients as per inception of the EMA Patient Registry Initiative, and according to the FAIR Principles.



EMA, European Medicines Agency; FAIR, findable, accessible, interoperable, reusable; HTA, health technology assessment.

References: Cave A, et al. Clin Pharmacol Ther. 2019;106:36-9. Walter MC, et al. J Neuromuscul Dis. 2019;6:453-65. Pechmann A, et al. Orphanet J Rare Dis. 2019;14:18. Mercuri E, et al. Neuromuscul Disord. 2019;29:794-9. The EMA Patient Registries Initiative: https://www.ema.europa.eu/en/human-regulatory/post-authorisation/patient-registries.

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