

2023

International Patient Advocacy Group Meeting



WELCOME AND INTRODUCTION

Kenneth Hobby, Cure SMA, USA

Meeting Goals

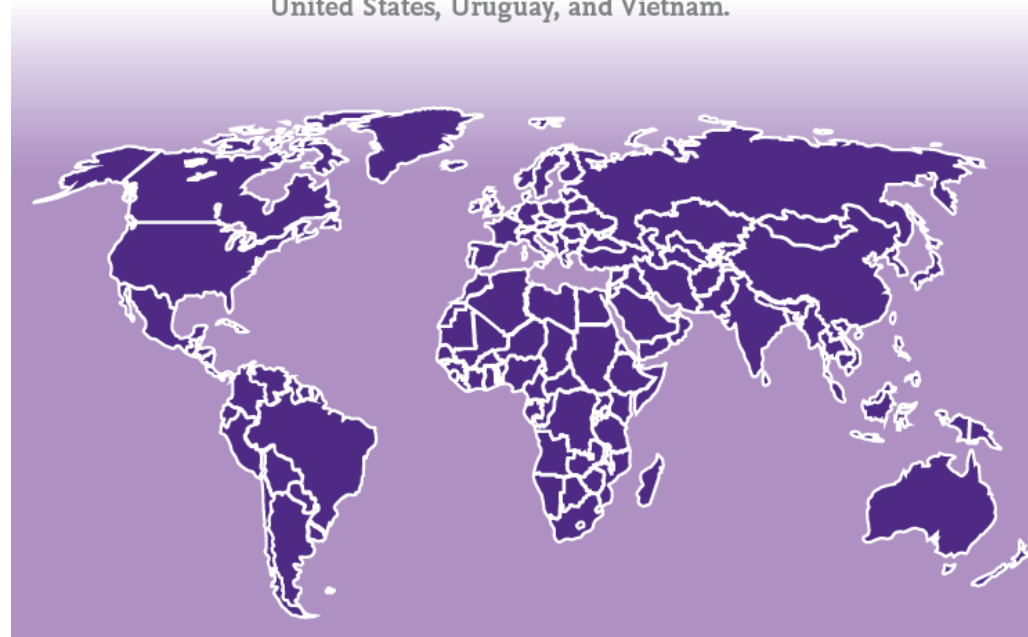
- **Informal First Try**
- **Will Record**
 - Translate and share after
- **Propose Next Steps:**
 - Continue meeting annually?
 - Rotate around regions
 - Establish a more formal structure?
 - Prioritize goals
 - Improve:Diagnosis, Access, Care, Quality of Daily Living

Countries Represented

WELCOME!

**45 Groups from
35 Countries**

We are honored to have SMA families, researchers, and clinicians attend this conference from all over the world! There are individuals attending from 46 countries, including: Argentina, Australia, Austria, Bangladesh, Belgium, Brazil, Bulgaria, Canada, Chile, China, Colombia, Costa Rica, Czech Republic, Ecuador, Finland, France, Georgia, Germany, Hungary, Iceland, India, Ireland, Italy, Japan, Jordan, Malaysia, Mexico, Morocco, Netherlands, Norway, Pakistan, Panama, Paraguay, Peru, Poland, Portugal, Puerto Rico, Slovenia, Spain, Switzerland, Taiwan, Turkey, United Kingdom, United States, Uruguay, and Vietnam.



**Additional Side Meetings
The Rest of the Conference**

Sponsors



Biogen



NOVARTIS

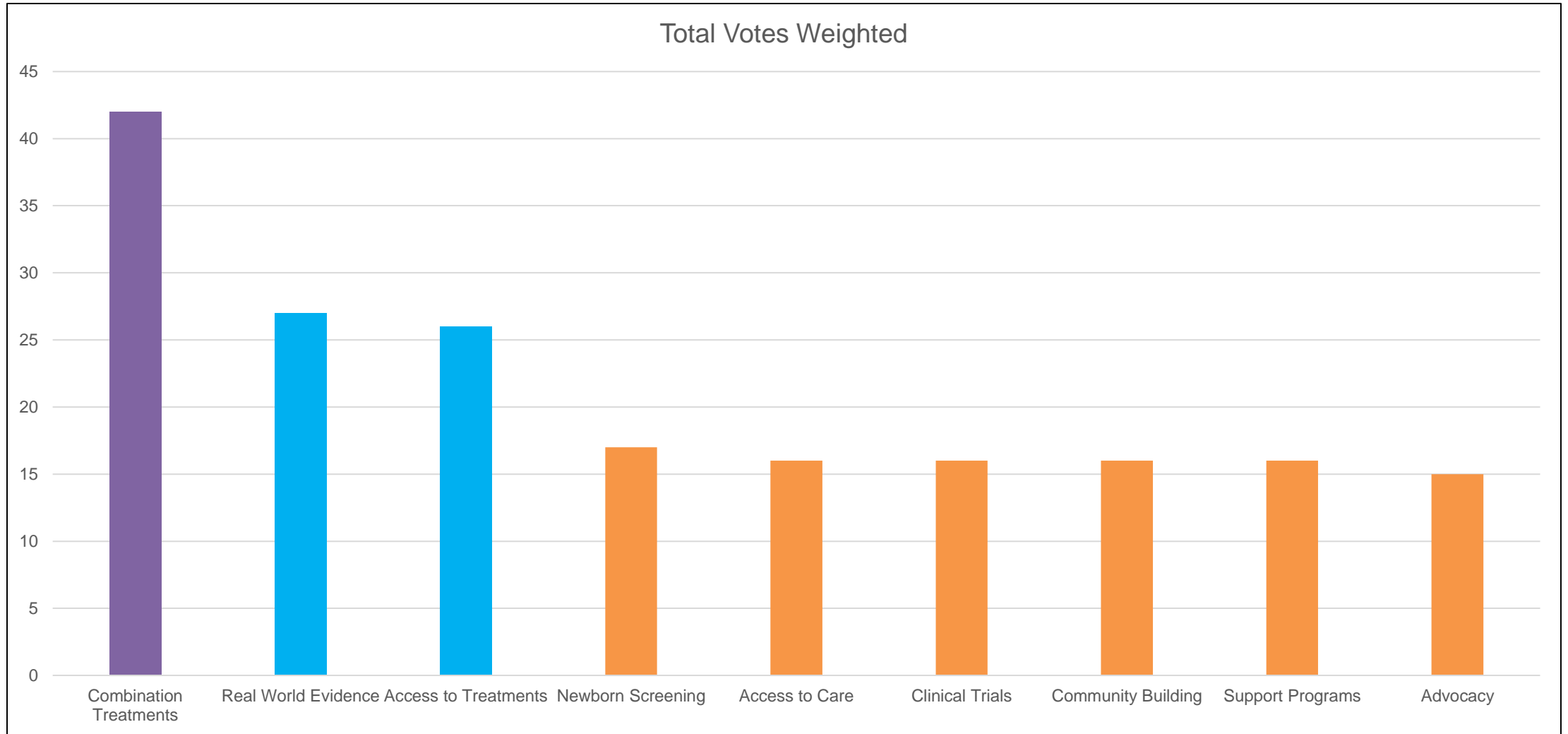


Roche

Agenda

- **8:30am – Breakfast**
- **9:00am – 9:20am - Welcome and Opening Session – Kenneth Hobby (USA)**
- **9:20am – 9:40am - SMA Europe Treatment Access Tools – Nicole Gusset (Europe)**
- **9:40am – 9:45am - Roche/Genentech Introduction**
- **9:45am – 10:05am - Care and Combination Treatment – Mary Schroth, MD (USA)**
- **10:05am – 10:10am - Novartis Gene Therapies Introduction**
- **10:10 am – 10:25am - Break**
- **10:25am – 10:45am - Real World Evidence and APAC – Julie Cini (Australia)**
- **10:45am – 10:50am - Biogen Introduction**
- **10:50am – 11:10am - Advocacy and NBS – Maynard Friesz (USA)**
- **11:10am – 11:30am - ALAME Coordination Across Countries and Groups and Differences –
Maria Isabel Acevedo, Vanina Sanchez (Latin America)**
- **11:30am – 11:50am - Challenges in Standards of Care and Access Wins –
Alpana Sharma, Moumita Ghosh (India)**
- **12:00pm - Lunch and Mingle**

Key Topics - Survey



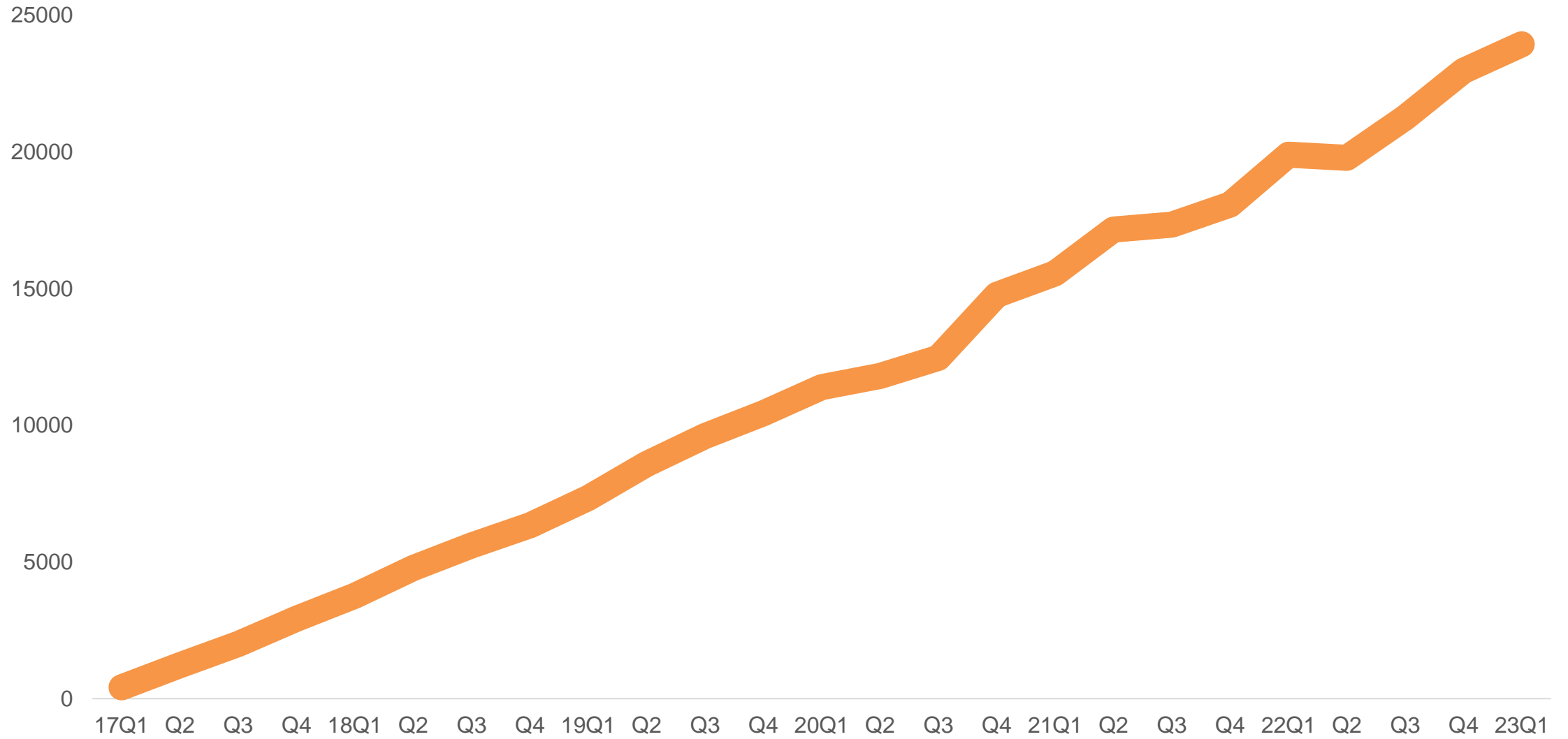
Quick Poll – Diagnosis and Treatment Access

- **Raise Hand**
- **Newborn Screening over 50% of births?**
- **No Treatment Approved/Available?**
- **One Treatment Approved/Available?**
- **Two Treatments Approved/Available?**
- **Three Treatments Approved/Available?**

Theme - SMA is the Same Worldwide

- **Same Genetics Worldwide**
 - **Same Treatment Impact Worldwide**
 - **Same benefits of Early Diagnosis Worldwide**
-
- **Estimated Prevalence of 225,000**

Total Patients on Treatment



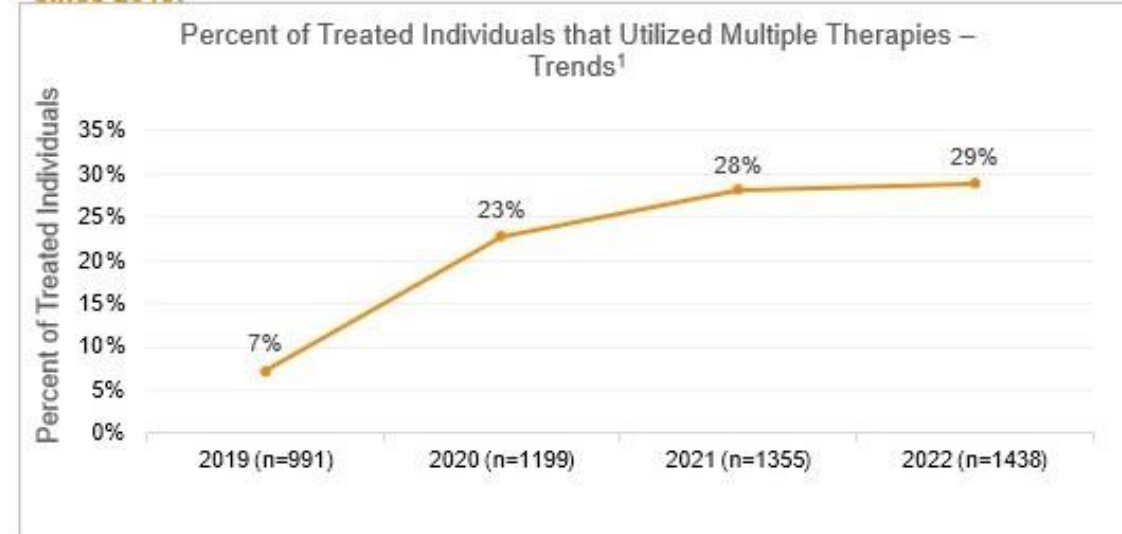
Processes to Access Treatments and Care

- **Different Country by Country**
 - Regulatory
 - Reimbursement/Payers
 - Healthcare Infrastructure
 - Delivery
 - Supportive Care
 - Safety and Efficacy
- **High Level of Variability**

Combination Treatments

- **Top Priority?**
 - Over single access...
- **Definitions**
 - Sequential/Switch
 - Concurrent/Same
- **Same Mechanism (SMN)**
- **Different Mechanisms (SMN/Muscle)**
- **Rationale for benefit?**
- **Data for benefit and safety and coverage?**

Approximately 30% of treated individuals have received 2 or more FDA approved SMA treatments and the use of multiple SMA treatments has risen since 2019.

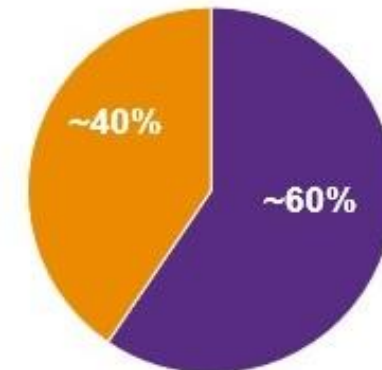


Approximately 40% of patients on multiple FDA approved treatments received overlapping treatments (>1 day) and 60% received treatments in a sequential manner.

Treatment Patterns for Patients who Received Multiple Treatments, n=378²

~ 40% of individuals that received multiple FDA approved SMA treatments indicated that the start date of one treatment was before the end date of another treatment.

Note: this includes all individuals who initiated a treatment after receiving Zolgensma.



~ 60% of individuals that received multiple FDA approved SMA treatments indicated that one treatment's end date was before the next treatment's start date.

Approach

- **Incremental Steps**
 - First Slow, then Stop, then Cure
- **Prioritize**
 - Treatment
 - Diagnosis
 - Care
 - Multiple Treatments
 - Combination Treatments

Cause

Effect

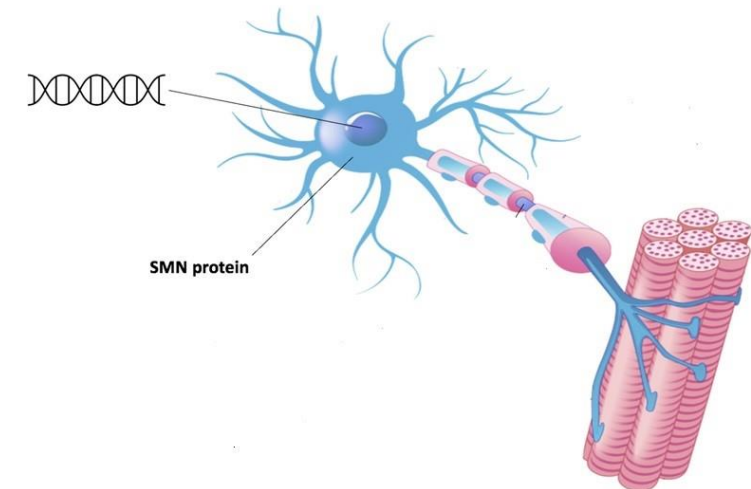


Early:
- NBS

First:
- **Genes/Protein**
- **Invisible**

Before Visible:
- Biomarkers

Then:
- Symptoms
- Visible
- Muscles





Future Steps

- **Survey**
- **Virtual Meeting**
 - 6 months
- **Annual Meeting**
 - 2024

- **Shared Resources/Data**

- **Countries/Regions/Global**
- **Medical Professionals/Industry**

- **Formal Structure**

- **Key Recommendation**
 - Pick and focus on just one or two priority issues to start