GNE Myopathy Global Registry & Natural History Study – A Model for Public/Private Collaboration

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Agenda

• Ultragenyx & TREAT-NMD
• Goals for the program
• Outline of the Patient Monitoring Program (PMP)
• Key features of the PMP
• Organizational structure
• Partnership
• Responsibilities of each Partner
• Summary
The Private & the Public

• Ultragenyx Pharmaceutical Inc.
  – Focused on rare and ultra rare disorders
  – Experienced management team (BioMarin, Genzyme, Elan, Janssen, Genentech, TKT, Enobia, Merck, Amgen)
  – Lead products are SA-ER for GNE myopathy and ERT for MPS 7 (Sly syndrome); other products in preclinical development

• TREAT-NMD
  – Recognized leader in neuromuscular disorders
  – Experienced in coordinating global registries
  – UK site for Natural History study (NHx)
Goals for the Program

• GNE Myopathy (aka: HIBM) is a neuromuscular disorder caused by a defect in the biosynthetic pathway for sialic acid (SA)
  − HIBM is a severe progressive myopathy that typically presents in early adulthood as weakness in the distal muscles of the lower extremities and progresses proximally, leading to a loss of muscle strength and function, and ultimately a wheelchair-bound state. There is no approved treatment for this disease.

• Natural History Program (NHx)
  − Given the rarity of the disease and the potential for novel therapies, there is a need to characterize the clinical presentation and progression of disease to support treatment development (especially for biomarker identification)
  − FDA/EMA requirement

• Registry
  − Identify potential patients for trial readiness and NHx recruitment
  − Locate and communicate with key physicians and researchers
  − Collaborate with or help to establish patient societies/support groups
  − Create a global public resource for HIBM research
The Patient Monitoring Program (PMP)

• A single integrated database combining patient reported (PRO) registry data and GCP quality natural history data

• Scalable architecture able to add multiple disease states in a dynamic relational database

• Improve the cost equation thru study design, frequency and quality control of data collection and verification

• Public/Private governance with defined charter and steering committee comprised of academic, biotech and patient leaders
Keys to the HIBM-PMP

• A web-based electronic data capture system and management platform for two components:
  – HIBM Disease Registry
    • Patient-reported data entered online is confirmed by physicians
  – HIBM Natural History Study
    • Study to assess muscle strength/function, biomarkers and patient reported outcomes

• A combination of GCP and non-GCP data from patients, physicians and investigators
  • Each data field qualified with a metadata tag as either unverified, physician verified, GCP or locked

• Role-based access with pre-defined reports available for scientific use by patients, physicians, and investigators
  • Access to data governed by a steering committee
HIBM-PMP Organizational Structure and Interface with Patients, Physicians & Researchers

- **Ultragenyx (Private Corporation)**
- **Treat NMD (Academic Institution)**
- **HIBM Steering Committee**
  - **HIBM Expert**
  - **Patient Group Leader**
  - **Researcher**
  - **Clinical Site**

**Database**
- **Registry/Natural History Study**
  - **GCP Data/Monitoring**
  - **Data Structure & Protocol Oversight**

**Processes**
- **Collaboration**
- **Requests**
- **Approval/Access**

**Interfaces**
- **Web based interface**
- **Patient info**
- **Research Reports**
- **Investigator Reports**
- **Sponsor Reports**

**Roles**
- **Patients**
- **MD’s**
  - **Physician Reports**
  - **Data Verification**
- **Web based interface**
Public/Private Partnership

• Form a Joint Project Team (JPT) with academic experts in disease state (TREAT-NMD)
• Provide initial funding for dedicated staff
• Collaborate on protocols for studies
• Identify & recruit steering committee
• Identify, recruit & qualify NHx sites
• Launch disease portal
  – Design interface & reports for PI and Patient
• Create public access to registry data (via Steering Cmte) thru joint ownership with TNMD
Responsibilities of Ultragenyx/Sponsor

- Provide initial funding for program – long term funding if successful development program
- Establish and oversee initial systems development with input from TNMD
- Select and pay for CRO for NHx
- Provide representatives for Joint Program Team
- Patent filings and prosecution (if any)
Responsibilities of TREAT-NMD/Partner

• Hire and oversee dedicated staff to program
• Manage steering committee with JPT
• Provide budgets and plans for agreed functions
• Provide scientific and medical expertise
• Provide access to TNMD network
• Provide tools, endpoints, know-how to assure effective conduct of the study
• Support surveys and outreach to identify patients & physicians and introduce them to the program
• Provide genetic testing
Summary

• The HIBM-PMP program is a unique, web-based, patient program that integrates a global registry and a GCP natural history study AND

• A public-private partnership with access to data for the patients, physicians and the research community

• The primary goals of the HIBM-PMP program are:
  – Increase understanding of the clinical presentation and progression of HIBM disease
  – Identify patients, facilitate early diagnosis and eventually optimize patient management
  – Identify and validate biomarkers and other HIBM specific efficacy measures

• HIBM-PMP program launch is planned in 4Q 2012

• For additional details please contact: http://clinicaltrials.gov or John Ditton jditton@ultragenyx.com
Questions?