Partnerships with Patient Advocacy Groups (PAGs) to advance Clinical development programs: Spotlight to Duchenne Muscular Dystrophy (DMD).

Vanessa Ferrera PhD, MPA (Santhera Pharmaceuticals). This project was completed in collaboration with DMD Patient Advocacy Groups (PAGs). Poster presented at 10th European Conference on Rare Diseases & Orphan Products (ECORD) (14-15 May 2003).

01 Introduction

What is Duchenne Muscular Dystrophy (DMD)?

DMD is a genetic, degenerative disease that is inherited in an X-linked recessive mode with an incidence of up to 1 in 3,500 live born males worldwide.1-3 DMD causes a progressive loss of muscle strength since boys diagnosed with DMD have a lack of a protein called dystrophin. Dystrophin protects muscle fibers from breaking down. Usually the leg muscles are affected first, resulting in children having the ability to walk by the age of 7-12 years. As boys grow older, the heart and respiratory muscles are affected.4

Where do we stand in terms of clinical research in the field of DMD?

Clinical research investigating potential treatments for DMD is a rapidly changing field. Currently, the Duchenne Drug Development Pipeline is composed of 39 potential treatments that are being tested.4 Among them, the SIDEROS trial: SIDEROS is a phase III clinical trial, evaluating the efficacy of the investigational drug ibeponex compared to a placebo, in delaying the loss of respiratory function in patients with DMD receiving glucocorticoids4-8.

Why is respiratory health important in DMD?

Duchenne affects muscles — including those important for breathing. Lung muscles will get weaker as the patient grows older. Patients with DMD are at high risk of respiratory complications as their clinical condition deteriorates due to progressive loss of respiratory muscle strength. Indeed, respiratory complications are a major cause of morbidity and mortality in DMD.4-8

Which respiratory symptoms may DMD boys experience?

- Inability to cough properly
- Fatigue and/or problem concentrating
- Frequent chest infection
- Morning headaches
- If a DMD patient experience any of these warning signs of respiratory dysfunction, a pulmonologist should be notified.

What was the purpose of this project?

This study aimed at providing:
- An understanding of motivations and barriers that influence participating in clinical trials;
- An understanding of the patient-friendliness of information on clinical trials in DMD (Spotlight on SIDEROS trial).

02 Materials and Methods

18 participants from 16 countries - Participants’ countries of origin included: Australia (n=1), Austria (n=1), Czech Republic (n=1), Germany (n=1), Greece (n=1), Hungary (n=1), Ireland (n=1), Italy (n=1), Poland (n=1), Portugal (n=1), Romania (n=1), Spain (n=1), Sweden (n=1), Switzerland (n=1), The United Kingdom (n=1), and the United States of America (n=1).

Methods - Quantitative research: 1) A survey was designed; 2) The data was collected; 3) Questionnaire data was analysed using descriptive statistical presentations, with the assistance of the software Survey Monkey.

03 Results - General information about the participation in clinical trials on DMD

The results show that nine out of eighteen participants were involved in designing and/or planning of DMD clinical trials.

- Identification of the outcome measures in clinical trials (N=9);
- Defining the most relevant research question (N=9);
- Identifying appropriate and ethically acceptable research tools and methods (N=8);
- Recruiting patients into clinical trial (N=8);
- Developing a clinical trial protocol (N=7);
- Providing a perspective on the interpretation of findings (N=7);
- Disseminating the results to ensure awareness of study findings and benefits (N=7);
- Measuring the impact of findings and informing future trial design (N=6);
- Access to transportation, drug side-effects and being referred to a trial site were considered the main barriers to participating in clinical trials (N=8).

04 Results - Resources to disseminate ongoing clinical trials

Internet and patient organisations were considered the main sources of information about existing clinical trials.

- Internet (n=17);
- Radio/television (n=15);
- Social media (n=11);
- Medical staff (n=10);
- Pharmacists/industry (n=10);
- Other healthcare professionals with the condition (n=9);
- Well-known group or patient group (n=8);
- Health boards/Nurse (n=7);
- Other (n=6).

Some DMD patients and/or families are still somewhat unaware about clinical trials (N=18).

05 Results - Health information related to the existing SIDEROS study of material

The information regarding the SIDEROS trial was considered fairly easy or very easy to understand.

06 Resources

- To learn about respiratory symptoms, management & care:
  - Oribanq and the World Wide Web is a key tool in providing information to people affected by DMD
  - Patient organizations’ preferred tools and materials to provide information about respiratory function in the muscles and medical, scientific conferences, interactions, group meetings, online forums and local media.

Duchenne Muscular Dystrophy News includes:
- Providing accurate and up-to-date information about Duchenne muscular dystrophy.
- SIDEROS study: evaluating the effectiveness of an investigational drug ibeponex in delaying the loss of respiratory function in patients with DMD receiving glucocorticoids.

07 Listening to the patient voice

It’s very good to see a trial open to adults, this is quite rare and means we haven’t been very involved with most pharmaceutical companies involved.

08 Conclusions

- The results of this study show the willingness of patients/caregivers to participate in designing and raising awareness for clinical trials.
- The findings also show the key role of patient organizations as a bridge for clinical trial dissemination.

09 References