Report CAB 2018/2019

DUCHENNE

COMMUNITY ADVISORY BOARD

Just over one year ago, WDO initiated the Duchenne Community Advisory Board (CAB) as part of the EuroCAB programme run by EURORDIS. It is an independent international board of 11 patient representatives from 11 different countries: UK, Ireland, France, Spain, Belgium, Netherlands, Germany, Italy, Rumania, Turkey and US. The CAB members represent the Duchenne community as a whole, neither their own child nor just their own country.

The goal of the Duchenne CAB is to meet with companies and give them its combined expert knowledge and advice on research goals, clinical trial protocols, informed consent forms, patient-relevant endpoints and much more in order, among other things, to optimize clinical trials for better and faster results, reduce the burden on patients and their families, enhance the clinical trial experience and then accompany the drugs through market approval and help bring them to the patients as quickly as possible.

The first meeting was held in Amsterdam in June 2018 with four companies that already have drugs for DMD in various clinical trial phases. We talked with all four about what kinds of endpoints are relevant to DMD patients and their families and what these mean in terms of activities of daily living and quality of life, as well as about the burden of clinical trials and how these might be reduced. To give a concrete example, we were able to convince one company to reduce the number of biopsies to be taken during a trial from 6 to 3. Another outcome was that we reviewed informed consent forms in an attempt to make them easier to understand for patients and parents.

Two of the same companies came back to the second Duchenne CAB meeting in November 2018 to continue what they clearly saw as a fruitful collaboration worth continuing and intensifying, as well as a new company hoping to start a first trial soon. This company is coming again to the next meeting in May 2019, when we hope to find out how much of our advice has been taken on board in the clinical trial protocol.

In May 2019 all companies we have seen so far are returning, as well as one new one. The rapid progress in the Duchenne field made it necessary to accept all 6 companies for the May session in order to stay on top of all new developments. However, the intention in general is to have no more than four at one CAB session.

The Duchenne CAB is committed to its aim of providing a “safe harbour” for confidential discussions with companies in an atmosphere of trust and respect for the ultimate benefit of the whole Duchenne community.