

5th Duchenne CAB Meeting May 2020

The Duchenne CAB held its 5th meeting from 13 – 15 May 2020. Due to the COVID-19 pandemic the meetings had to be held virtually via video conference. Due to time differences, the conferences could only be held during the afternoon, so we were only able to accommodate three companies during this period; one virtual meeting was held on 28 May, and one has been postponed indefinitely.

Some of the main topics discussed

- Clinical trials in the non-ambulant DMD population including the particular challenges and burdens in these more fragile patients
- PUL 2.0, FVC%p and cardiac MRI as viable endpoints acceptable to regulators
- The burden of biopsies and the option of forgoing baseline biopsies since it is well known that only small amounts of dystrophin are found in DMD patients
- The advantages of using a DMD-specific quality of life questionnaire (DMD-QoL) developed by the UK based HERCULES Project, in order to demonstrate its reliability and sensitivity to regulators and payers, in the hope that it may eventually replace the currently used, non-specific EQ-5D
- Placebo and randomization ratios
- The current lack of up-to-date Natural History data in the non-ambulant DMD population
- The need to share placebo data between companies in a timely manner
- CROs and the initiation of “concierge” services to help guide patients and their families through the clinical trial experience

In between these meetings there are frequent follow-up calls and communication both between CAB and companies and internally

In addition, the Duchenne CAB is constantly enhancing its knowledge by regular training following the [EURORDIS Summer and Winter School](#) programs, and a number of CAB members have now completed the face-to-face, or this year, the virtual, Summer School and Winter School programs, as well as the [European Patients Academy \(EUPATI\)](#) patient expert training course.

Announcement

Next Duchenne CAB dates for 2020: 7 – 10 October 2020. As a result of the continuing uncertainties around the COVID-19 situation, these meetings will also be virtual.

Inquiries

If you have any questions concerning the Duchenne CAB or any issues you would like to bring to our attention, please contact the Duchenne CAB Coordinator: sally@duchennedatafoundation.org