

**Orphazyme Statement on NCT02753530:
Study of Arimoclomol in Inclusion Body Myositis (IBM)**

In late 2017, Orphazyme formally assumed sponsorship of the [Phase II/III Study of Arimoclomol in sporadic Inclusion Body Myositis \(sIBM\)](#) from Kansas University Medical Center. The purpose of the study is to evaluate the safety and efficacy of arimoclomol in people living with sIBM.

The multi-center trial began enrolling participants in August 2017 in the USA and in August 2018 in Europe. Thanks to strong interest from the IBM community, Orphazyme is pleased to announce the completion of enrollment, having reached our goal of 150 participants across 12 study locations.

The study is a 20-month trial comparing placebo with active treatment (arimoclomol) with a planned interim analysis at 12 months. However, it is not anticipated that the study will end at this time. The final study results are expected in the first half of 2021 at the conclusion of the 20-month timepoint. The 20-month primary analysis time-point was established with regulatory authorities (FDA and EMA) to maximize chances of success, potentially allowing for a meaningful difference to be seen between participants receiving active treatment vs placebo.

Our team looks forward to continued conversations with The Myositis Association (TMA), Myositis Support & Understanding (MSU), the Muscular Dystrophy Association (MDA), those participating in the Yale IBM Registry, and other patient organizations to ensure the latest information regarding Orphazyme's clinical development program in sIBM is available to those who need it most: People living with sIBM and their loved ones.

Orphazyme would like to express our sincere appreciation to those who have shown interest in the study. The speedy trial enrollment has brought us a significant step closer to understanding whether arimoclomol could potentially be a safe and effective treatment option for those living with sIBM. Thank you for your support of our efforts to develop a therapy for this devastating disease.