Biophytis is developing BIO101 for DMD. The pre-clinical proof-of-concept (PoC) for BIO101 suggests a range of benefit in a broad DMD population efficient for study site staff. To ensure an optimal and patient-centered clinical development of BIO101 a seamless methodology will be utilized.

There is a significant need, therefore, for utilization of novel designs, which will make clinical development more approachable for patients and more efficient for study site staff. Biophytis is developing BIO101 for DMD. The pre-clinical proof-of-concept (PoC) for BIO101 suggests a range of benefit in a broad DMD population efficient for study site staff.

The MYODA Seamless clinical trial design: A true innovation for Rare Diseases including DMD

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BACKGROUND

Clinical development of new medications is traditionally broken down into 3 phases. This prevents patients from participating in more than one of the development phases and comes with a burden on site staff (with repeated site initiation and training) leading to prolonged development timelines (up to 10 years) and difficulties in patient recruitment, especially in rare diseases such as Duchenne Muscular Dystrophy (DMD).

There is a significant need, therefore, for utilization of novel designs, which will make clinical development more approachable for patients and more efficient for study site staff.

The MYODA study is designed to address the challenges that medication development faces, especially in rare disease. It also aims to accommodate the needs of DMD patients while maximizing efficiency and operational excellence.

STUDY SCHEME

- The MYODA study is designed to address the challenges that medication development faces, especially in rare disease. It also aims to accommodate the needs of DMD patients while maximizing efficiency and operational excellence.
- The MYODA study design will also ascertain that it is conducted under the highest standards and scientific rigor. The study will be closely monitored by a data monitoring committee and the regulatory authorities in the countries where it is conducted.

STUDY POPULATION

Study inclusion is will be non-restrictive as possible. Patients with DMD, aged 7 and above and FVC <50%, ambulant and non-ambulant, will be included. Patients not eligible for the double-blind period of part 3, will be offered to participate in the open-label parallel arm of part 3.

ENDPOINTS AND ANALYSIS

The study population is broad and diverse and patients experience disability in an individual manner, there is a need to assess the efficacy of BIO101 in a manner that will reflect the individual experience.

See more details in the Composite Score poster# P11-128-#459 for more details.

DISCUSSION AND IMPLICATIONS

- The MYODA study is designed to address the challenges that medication development faces, especially in rare disease. It also aims to accommodate the needs of DMD patients while maximizing efficiency and operational excellence.
- The MYODA study design will also ascertain that it is conducted under the highest standards and scientific rigor. The study will be closely monitored by a data monitoring committee and the regulatory authorities in the countries where it is conducted.

DATA MONITORING COMMITTEE (DMC)

1. Begin recruitment to the 2mg/kg/d cohort
2. Begin recruitment to the 4mg/kg/d cohort; move subjects from the 2mg/kg/d cohort to the 5mg/kg/d cohort
3. Begin recruitment to the 5mg/kg/d cohort; move subjects from the 2mg/kg/d cohort to the 5mg/kg/d cohort
4. Move subjects from the 4mg/kg/d cohort to the 5mg/kg/d cohort; begin recruitment of additional subjects to part 2
5. End part 2; move all subjects from the 5mg/kg/d cohort to the open-label run-in period of part 3; begin recruitment to the open-label parallel arm
6. Begin randomization of subjects in the open-label run-in period of part 3; begin recruitment to the open-label parallel arm
7. End of study; move all subjects into the open-label extension

STUDY OBJECTIVE

- Design
- Period / element
- Subjects
- Length / timing

1. Safety, tolerability, pharmacokinetics (PK)
   - Multiple ascending doses, randomized, blinded, placebo-controlled
   - 4 cohorts, 12 subjects in each (3 placebo, 9 treatment)
   - 7 days

2. Proof-of-Concept (PoC) Biomarker and pharmacodynamics (PD)
   - Adaptive, randomized, blinded, placebo controlled, with a recurrent interim analysis to assess success and futility
   - All subjects, who participated in part 1 continue directly into part 2
   - Double-blind period
   - Assessment for success and futility is done every 3 months by the DMC
   - Subjects will remain on placebo for a maximum of one year, after which they will begin to receive study medication.
   - 5-12 months

3. Confirmatory – efficacy and safety
   - Parallel arms, randomized withdrawal, double-blind, placebo-controlled
   - The DMC will monitor continuously, to identify subjects who have deteriorated due to being randomized to placebo.
   - Randomization of subjects in the open-label run-in period of part 3; begin recruitment to the open-label parallel arm
   - Until approval

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