

## Biophytis receives Orphan Drug Designation from the FDA for Sarconeos in Duchenne Muscular Dystrophy (DMD)

Paris (France), May 15, 2018, 7.30 pm - BIOPHYTIS (Euronext Growth Paris: ALBPS), a biotechnology company specialized in the development of drug candidates to treat age-related degenerative diseases, announces that the FDA (Food and drug administration) has granted Orphan Drug Designation (ODD) to its drug candidate Sarconeos in Duchenne Muscular Dystrophy (DMD).

**Stanislas Veillet, CEO of BIOPHYTIS, said:** *"The Orphan Drug Designation that has just been granted by the FDA is a significant milestone for the development of Sarconeos in DMD, a particularly severe pediatric indication. We hope to obtain the ODD in Europe in the coming weeks. This will allow us to advance the clinical development program in DMD, entitled MYODA. This program would consist of two studies: a pharmacokinetic phase 1/2a MYODA-PK study, which could begin in 2018, and an efficacy phase 2/3 MYODA-INT study, which could start in 2019."*

The Orphan Drug Designation by the FDA will provide Sarconeos the following benefits in the USA in DMD indication: a 7 years exclusivity after marketing authorization, possible co-financing of clinical trials by the FDA, and the possibility of a fast track registration procedure. Biophytis has also filed an application for an Orphan Drug Designation for Sarconeos in DMD in Europe with the EMA (European Medicines Agency). The Agency's response is expected in the coming weeks.

Sarconeos' proof-of-concept in the treatment of Duchenne Muscular Dystrophy has been presented at the World Muscle Society congress in 2017. It was demonstrated, in the reference animal model of Duchenne Muscular Dystrophy, that Sarconeos had significantly improved exercise tolerance and muscle strength, and reduced muscle fibrosis. There are very few effective treatment options and Sarconeos is a new class of drug candidate that has the potential to significantly slow down the progression of the disease. It could be used as a standalone treatment, or in combination with gene therapy when it will be available for children with DMD.

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### About MYODA:

MYODA is the name of drug candidate Sarconeos' new clinical development program in Muscular Dystrophy or Duchenne myopathy (DMD). Sarconeos is a drug candidate that activates the MAS receptor, stimulates muscle anabolism and reduces the appearance of muscle fibrosis, with the potential to suspend the disease's progression, particularly to delay the loss of mobility. The clinical development program will include a phase I/II pharmacokinetic study (MYODA-PK), which is expected to begin in 2018, and a phase II/III study (MYODA-INT), which is expected to start in 2019.

### About Duchenne muscular dystrophy:

Duchenne muscular dystrophy (DMD), is an X-linked inherited muscular disease, which concerns 1 in 3,500 male births, and characterized by progressive muscle weakness and cardiomyopathy, leading to premature death. Muscles undergo repeated cycles of necrosis/regeneration and are replaced by

connective and adipose tissues. Glucocorticoids and supportive therapy are the current standard of care leaving many patients with an unmet medical need.

#### About SARCONEOS:

Sarconeos is a first-in-class drug candidate based on the activation of the MAS receptor (major player of the renin-angiotensin system) restoring muscular anabolism, inhibiting myostatin, and that had demonstrated meaningful activity in animal models of muscular dystrophies. Sarconeos is developed in the treatment of sarcopenia, an age-related degeneration of skeletal muscle, leading to loss of mobility in elderly people. This condition, for which no medical treatment currently exists, was first described in 1993 and has entered the International Classification of Diseases (M62.84) in 2016. It affects more than 50 million people worldwide.

#### About BIOPHYTIS

Biophytis SA ([www.biophytis.com](http://www.biophytis.com)), founded in 2006, develops drug candidates targeting diseases of aging. Using its technology and know-how, Biophytis has begun clinical development of innovative therapeutics to restore the muscular and visual functions in diseases with significant unmet medical needs. Specifically, the company is advancing two lead products into mid-stage clinical testing this year: Sarconeos (BIO101) to treat sarcopenic obesity and Macuneos (BIO201) to treat dry age-related macular degeneration (AMD).

The business model of BIOPHYTIS is to ensure the conduct of the project until clinical activity in the patient is proven, then to license the technologies in order to continue the development in partnership with a pharmaceutical laboratory.

Based on the Sorbonne Université campus, Biophytis collaborates with expert scientists from several Sorbonne Université institutes such as the Paris Seine Biology Institute, the Institute of Myology, and the Vision Institute.

BIOPHYTIS is listed on the Euronext Growth market of Euronext Paris (ALBPS; ISIN: FR0012816825).

For more information: <http://www.biophytis.com>

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of an offer to buy or subscribe to shares in BIOPHYTIS in any country. Items in this press release may contain forward-looking statements involving risks and uncertainties. The Company's actual results could differ substantially from those anticipated in these statements owing to various risk factors which are described in the Company's prospectus. This press release has been prepared in both French and English. In the event of any differences between the two texts, the French language version shall prevail.

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