SOM Biotech announces initiation of Phase 2a Clinical Trial of SOM3355 in Huntington's disease patients

Barcelona, Spain and Cambridge, MA, 17 July 2018—SOM Biotech, a clinical-stage biopharmaceutical company that discovers and develops drugs for orphan indications in the Central Nervous System, has announced today the successful initiation of Phase 2a clinical trial of its repurposed compound SOM3355 for the treatment of the chorea movements associated to Huntington's disease.

The Principal Investigator of the study is Dr. Jaume Kulisevsky from Hospital de la Santa Creu i Sant Pau, Barcelona, Spain. Other participating sites, also located in Barcelona, are Hospital Universitari de Bellvitge, Hospital Clínic de Barcelona and Hospital Universitari Vall d'Hebron, with leading neurologist investigators Dr. Matilde Calopa Garriga, Dr. Esteban Muñoz and Dr. Josep Gàmez, respectively.

The study is a Phase 2a proof-of-concept and is designed as a randomized, double-blind, placebo-controlled, 2-arm, 27-week study to evaluate the safety and efficacy of SOM3355 in Huntington's disease (HD) patients with chorea movements. A total of 30 patients will be involved and improvement in their Total Maximal Chorea score will be measured as the primary efficacy endpoint. The study will also incorporate measurements in the different domains of the disease such as impression of change, functional capacity, gait and motor scores, as well as safety, as secondary endpoints.

The investigational product, SOM3355, is a repositioned drug previously approved for another indication. It is a cardioselective β1-adrenoceptor antagonist discovered in US, launched in several European countries and is currently commercialized in some Asian countries for the treatment of hypertension and angina pectoris. SOM3355 is an oral and brain penetrant drug that was discovered by SOM Biotech to be a potent selective inhibitor of Vesicular Monoamine Transporter-2 (VMAT2), using its artificial intelligence genetic algorithm platform (Phoenix®). The molecule causes a decrease in the levels of Dopamine, which plays an essential role in the control of coordinated movements, thus reducing choreic movements associated with Huntington's disease. SOM has conducted numerous validating preclinical studies which thus far indicate that SOM3355 has the potential to have promising clinical efficacy as well as properties that are likely to avoid the side effects of other members of the VMAT2 inhibitor class that have led to restricted prescribing.

Huntington's disease (HD) is an orphan progressive neurodegenerative condition which demonstrates an autosomal dominant mode of inheritance. It includes a triad of motor, cognitive and psychiatric deficits. Chorea, an involuntary jerky movement that is purposeless and abrupt, is the most characteristic symptom of HD. HD predominantly affects the distal extremities and facial muscles. Abnormal movements usually become gradually more severe and many patients finish their years with very severe chorea that compromises motor function. According to SOM Biotech's internal assessment, this pathology has a

prevalence of 6-8/10^5 in western countries with a potential sales market of $700M WW. SOM3355 could also be extended to other related diseases such as Tourette syndrome, tardive dyskinesia and ballism.

"We are very proud to start this clinical trial of SOM3355 in Huntington patients. It will validate for the second time our business model and Phoenix® technology while providing patients with safer and more affordable drugs. The four hospitals taking part in the trial are of excellent profile and we are confident that the recruitment will go smoothly." said Dr. Raúl Insa, SOM Biotech Founder and CEO. Dr. Jaime Kulisevsky has added "Currently, the treatment of movement disorders in Huntington's disease is limited by the side effects of the drugs used. SOM3355 offers a promising alternative to improve the tools available to treat the disease. The clinical trial that we begin has a double-blind and randomized design that will allow to verify objectively the ability of the drug to improve abnormal movements. The participation of four centers in our environment will facilitate the recruitment of the sufficient number of patients and guarantee the objectivity of the results."

About SOM Biotech

SOM Biotech Ltd. (www.sombiotech.com) is a med-tech biopharmaceutical company founded in 2009 in Barcelona (Spain) with a business and expanding presence in Cambridge, Massachusetts (United States). The company's business strategy consists of identifying, clinically developing and marketing drugs to treat orphan indications in the Central Nervous System, using drugs already on the market (repurposing).

SOM has an extensive portfolio of products that includes drugs for rare diseases (Adrenoleukodystrophy, Adrenomyeloneuropathy and Duchenne Muscular Dystrophy) as well other drugs for different therapeutic areas. The company is also engaging in repurposing collaborations with major pharmaceutical companies by supporting them in developing the drugs in their portfolios and through partnership agreements.

SOM Biotech is a private company with international health sector professionals as investors on board. Since its inception in 2009, the company has raised more than €6M from private capital, and from public grants and loans. The company is getting prepared consciously to going public in approximately 2 years' time.

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