June 27th, 2023

HL001 receives Orphan Drug Designation from US FDA

YAMAGUCHI and KYOTO, JAPAN, June 27th, 2023 – UBE Corporation (Headquarter: Ube, Yamaguchi; President Masato Izumihara; hereinafter "UBE") and HiLung Inc. (Headquarter: Sakyo-ku, Kyoto; Chief Executive Officer: Yuki Yamamoto; hereinafter "HiLung"), have received Orphan Drug Designation (ODD) from US FDA (Food and Drug Administration) for HL001 – a novel lysophosphatidic acid receptor-1 (LPA1) antagonist under joint development for idiopathic pulmonary fibrosis (IPF).

IPF is an increasingly-debilitating chronic disease characterized by fibrosing stiff lungs and diminishing lung vital capacity, leading to an average mortality of over 50% in five years after diagnosis – the course of which has been overwhelmingly irreversible. HL001 is a promising drug candidate supported by a public R&D grant from AMED (Japan Agency for Medical Research and Development – the governmental funding agency for health sciences) with its "Support Program for Orphan drug prior to Designation intended for likely orphan therapeutics. US FDA has now granted ODD for HL001, based on preclinical datasets we submitted demonstrating plausible efficacy, including results from our unique *in vitro* pulmonary fibrosis assay that replicates pathologic shrinking and curative re-expansion in human induced pluripotent stem cell (iPSC)-derived alveolar organoids. This Designation allows for preferential incentives and R&D support from the FDA and other Federal Agencies, including a waiver of User Fee charged upon NDA (New Drug Application), deduction of Federal tax for clinical development, and a potential seven-year exclusivity once the drug is approved. These and other forms of support from public agencies will further accelerate the development of HL001, by facilitating multi-national, multi-institutional clinical trials that would be critical in drug development for rare diseases.

UBE and HiLung had entered into a joint research agreement in March 2021, and a joint development agreement in March 2022, and have strived to turn HL001 into a success, by leveraging UBE's drug discovery and chemical engineering capabilities, and HiLung's expertise in pulmonology and respiratory biology, including iPSC-derived respiratory cell models. Our aspirations include development of a patient-centric solution that will offer daily accessibility for recurrent use in IPF patients, many of whom unfortunately face chronic sequelae. We believe this ODD is a meaningful step forward that demonstrates the validity of our vision and our technologies in realizing patient value, and we will continue to make progress towards clinical trials, and subsequent clinical use.

Building on this regulatory Designation in the US, the two companies will deepen existing collaborations, will accelerate development and global licensing of HL001, and will continue to strive to offer patients a valuable solution to IPF.

Reference:
Ube Industries and HiLung Sign Joint Development Agreement for Co-Development of Novel Selective LPA1 Antagonist HL001 for Pulmonary Fibrosis (March 28th, 2022)

About UBE Corporation
UBE Corporation encompasses a group of specialty chemicals businesses, of which the pharmaceuticals business comprises the core of its life sciences portfolio, progressing beyond its track record of discoveries in small molecule therapeutics into high added-value products such as ADCs (antibody-drug conjugates). Alongside is a CDMO (contract development and manufacturing organization) business, which is strengthening its existing small molecule production capacity while also acquiring capabilities in novel modalities such as oligonucleotide therapeutics. UBE's life science businesses will continue to offer solutions that enhance and protect human life and health. https://www.ube.co.jp

About HiLung Inc.
HiLung was founded in 2020 to dramatically accelerate respiratory drug discovery and development, based on pioneering cell engineering technologies which reproducibly and scalably mass-produce respiratory cells from human stem cells, including for high-throughput purposes. We are offering assaying services based on human respiratory disease models, lung toxicity models, and respiratory infection models (incl. COVID-19) that replicate human-specific pathologies – all leading to human(e), non-animal, target identification, lead optimization, and patient stratification studies. These studies and resulting pipeline development not only serve the patients directly, but will also be foundational building blocks for our ultimate dream of regenerative lung transplantation therapy. In other words, we offer "human(e) resolution from human complexity".
"Translating Human Inspirations." – https://www.hilung.com/en/

For further inquiries:
UBE Corporation: Contact Form
https://www.ube.co.jp/ube/Inquiry/Form/InquiryIrprEnAgree

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Note: HL001 is an investigational compound and their human therapeutic efficacy and safety have not been clinically established.