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Company Name: AnGes Inc.
Presentative: Ei Yamada, President & CEO

Notice of Orphan Drug Designation for lonafarnib (U.S. brand name: Zokinvy) for the treatment of premature aging (HGPS and PL)

AnGes is pleased to announce that lonafarnib (U.S. brand name: Zokinvy), a drug for the treatment of Hutchinson-Gilford progeria syndrome (HGPS) and processing-deficient progeroid laminopathy (PL), for which we are preparing to submit an application for early approval, has been granted orphan drug designation by the Japanese Ministry of Health, Labour and Welfare.

1. About Zokinvy

Zokinvy was approved in the US in November 2020 for the treatment of HGPS and PL with processing failure, marketed by Eiger BioPharmaceuticals Inc in the US. In May 2022, AnGes signed an exclusive distribution agreement with the company for the Japanese market.

Zokinvy inhibits the accumulation of farnesylated mutant proteins (which induce cell destabilization and premature senescence) that form tight junctions with the nuclear membrane in patients with HGPS and processing-deficient PL.

Zokinvy reduced mortality by 60% (p=0.006) and prolonged mean survival by 2.5 years (p=0.005) in pediatric and young adult patients with HGPS. The most commonly reported side effects were gastrointestinal (vomiting, diarrhea, nausea), most of which were mild or moderate (grade 1 or 2). Many HGPS and dysprocessing PL patients treated with Zokinvy have continued treatment with Zokinvy for more than 10 years.

2. About an Orphan Drug

The Ministry of Health, Labour and Welfare (MHLW) designates orphan drugs on the condition that the number of eligible patients in Japan is less than 50,000 and that there is a particular medical need for the drug as a means of treating a serious disease.

Once an Orphan Drug Designation is obtained, the applicant is entitled to benefits and support measures such as priority review and a 10-year re-examination period if the product is approved for the designated indication.

3. Future outlook

We will not incur any special costs for this Orphan Drug Designation. We plan to promptly announce any events that should be disclosed in the future.