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Pfizer, Repligen to push spinal muscular atrophy program

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Pfizer, Repligen sign agreement for spinal muscular atrophy program

Singapore: US-based Repligen has entered into an exclusive worldwide licensing agreement with Pfizer to advance Repligen's spinal muscular atrophy (SMA) program, originally in-licensed from Families of SMA (FSMA).

The SMA program includes RG3039, a small molecule drug candidate in clinical development for SMA, as well as back-up compounds and enabling technologies. Repligen will receive up to $70 million from Pfizer, commencing with an upfront payment of $5 million and total potential future milestone payments of up to $65 million as well as royalties on any future sales of SMA compounds developed under the agreement. SMA is an orphan neurodegenerative genetic disease that presents early in life.

"This agreement is consistent with the strategic decision we announced in August 2012 to focus Repligen's internal efforts on the growth of our bioprocessing business, while seeking external partners for our therapeutic development programs," said Dr Walter C Herlihy, president and chief executive officer of Repligen. "We believe this collaboration with Pfizer, a leading pharmaceutical company with specialized efforts in orphan and genetic diseases, has the potential to accelerate the development of therapies for SMA."

"There is a critical need to expedite potential treatment solutions for rare diseases such as spinal muscular atrophy, where patients have such limited options," said Mr Jose Carlos Gutierrez-Ramos, senior vice president, Pfizer BioTherapeutics R&D. "This partnership will combine our expert capabilities in advancing molecules for genetic diseases with Repligen's leading SMA program."

Repligen will be responsible for completing the first two cohorts of an active phase I trial evaluating RG3039 in healthy volunteers, which it anticipates will occur during the first quarter of 2013. Repligen will also provide certain technology transfer services to Pfizer who will then assume full responsibility for the SMA program moving forward, including the conduct of any registration trials necessary for product approval.

Repligen has previously received US Orphan Drug and Fast Track designations for RG3039 for the treatment of SMA, as well

as Orphan Medicinal Product designation in the EU.