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Aptorum Group Receives FDA Orphan Drug Designation for its SACT-1 Repurposed Drug For The Treatment of Neuroblastoma

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NEW YORK & LONDON & PARIS--(BUSINESS WIRE)--Regulatory News:

Aptorum Group Limited (Nasdaq: APM, Euronext Paris: APM) ("Aptorum Group" or "Aptorum"), a clinical-stage biopharmaceutical company, is pleased to announce that the United States Food and Drug Administration (FDA) Office has granted Orphan Drug Designation to SACT-1, a repurposed small molecule compound for the treatment of patients with Neuroblastoma. Aptorum Group plans to file an Investigational New Drug Application (IND) to commence a phase 1b/2a clinical trial for SACT-1 to test the drug in neuroblastoma patients in 2022.

Mr. Darren Lui, President and Executive Director of Aptorum Group says, "The granting of orphan drug designation for SACT-1 for the treatment of neuroblastoma is another important step forward in the development of our drug candidate and reflects both the FDA's and Aptorum's commitment to addressing the unmet clinical needs of patients with neuroblastoma." Further to our recently announced completion of Phase 1 clinical trial and patent grant for SACT-1, we are currently focusing on our IND preparation for entering into the exciting Phase Ib/2a clinical trials for SACT-1 in the United States."

About SACT-1

SACT-1 is an orally administered repurposed small molecule drug to target neuroblastoma. SACT-1's mechanism has been investigated in our preclinical studies to enhance tumor cell death and suppress MYCN expression (a common clinical diagnosis in high-risk or relapsed neuroblastoma patients where an amplification of MYCN is usually observed). SACT-1 is designed to be used especially in combination with standard-of-care chemotherapy.

About Neuroblastoma

Neuroblastoma is one of the most prevailing solid tumor cancers in children, representing 8% - 10% of all childhood tumors, accounting for c. 15% of all cancer related deaths in the pediatric population[1]. For the high-risk patient group, the 5-year survival rate of this condition is around 40-50% as observed by the American Cancer Society[2] based on existing treatment.

[1] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3668791/#:~:text=Neuroblastoma%20is%20the%20most%20common,deaths%2(
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[2] https://www.cancer.org/cancer/neuroblastoma/detection-diagnosis-staging/survival-rates.html

About Aptorum Group Limited

Aptorum Group Limited (Nasdaq: APM, Euronext Paris: APM) is a clinical stage biopharmaceutical company dedicated to the discovery, development and commercialization of therapeutic assets to treat diseases with unmet medical needs, particularly in oncology (including orphan oncology indications) and infectious diseases. The pipeline of Aptorum is also enriched through (i) the establishment of drug discovery platforms that enable the discovery of new therapeutics assets through, e.g. systematic screening of existing approved drug molecules, and microbiome-based research platform for treatments of metabolic diseases; and (ii) the co-development of a novel molecular-based rapid pathogen identification and detection diagnostics technology with Accelerate Technologies Pte Ltd, commercialization arm of the Singapore's Agency for Science, Technology and Research.

For more information about Aptorum Group, please visit www.aptorumgroup.com.

Disclaimer and Forward-Looking Statements

This press release does not constitute an offer to sell or a solicitation of offers to buy any securities of Aptorum Group.

This press release includes statements concerning Aptorum Group Limited and its future expectations, plans and prospects that constitute "forward-looking statements" within the meaning of the US Private Securities Litigation Reform Act of 1995. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "estimates," "predicts," "potential," or "continue," or the negative of these terms or other similar expressions. Aptorum Group has based these forward-looking statements, which include statements regarding projected timelines for application submissions and trials, largely on its current expectations and projections about future events and trends that it believes may affect its business, financial condition and results of operations. These forward-looking statements speak only as of the date of this press release and are subject to a number of risks, uncertainties and assumptions including, without limitation, risks related to its announced management and organizational changes, the continued service and availability of key personnel, its ability to expand its product assortments by offering additional products for additional consumer segments, development results, the company's anticipated growth strategies, anticipated trends and challenges in its business, and its expectations regarding, and the stability of, its supply chain, and the risks more fully described in Aptorum Group's Form 20-F and other filings that Aptorum Group may make with the SEC in the future, as well as the prospectus that received the French Autorité des Marchés Financiers visa n°20-352 on 16 July 2020. As a result, the projections included in such forward-looking statements are subject to change and actual results may differ materially from those described herein.

Aptorum Group assumes no obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

This announcement is not a prospectus within the meaning of the Regulation (EU) n°2017/1129 of 14 June 2017 as amended by Regulations Delegated (EU) n°2019/980 of 14 March 2019 and n°2019/979 of 14 March 2019.

This press release is provided "as is" without any representation or warranty of any kind.

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