

Aptorum Group Announces Further Positive Data on SACT-1 Against Neuroblastoma and Other Potential Tumor Types

LONDON--([BUSINESS WIRE](#))-- Regulatory News:

Aptorum Group Limited (NASDAQ: APM, Euronext Paris: APM) (“Aptorum Group”), a biopharmaceutical company focused on the development of novel therapeutics including orphan diseases and oncology indications, announced further positive data from its latest *in vivo* studies showing significant activity against neuroblastoma tumor reduction when treated with its lead compound SACT-1 in combination with standard of care (SOC) chemotherapy. Separately, SACT-1 was also screened for its *in vitro* activity against over 300 cancer cell lines and showed positive results in a number of cancer types including in particular colorectal cancer, leukemia and lymphoma, etc.

Our repurposed drug candidate, SACT-1 is undergoing preparation for IND submission and is on track for regulatory application to target to commence phase 1b/2a clinical trials under the US FDA’s 505(b)(2) pathway.

“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 population¹. For the high-risk patient group, the 5-year survival rate of this condition is around 40-50% as observed by the American Cancer Society² based on existing treatment. We are delighted to see the progress of our SACT-1, one of our first assets from our SMART-ACT[®] platform. We are extremely excited to observe SACT-1’s significant effect on tumor shrinkage when used in combination with standard of care chemotherapy in our latest *in vivo* studies. Moreover, we believe that SACT-1 may have potential applications in a number of other cancer types, including non-orphan cancers, which we will be continuing to investigate further for its wider application,” said Dr. Clark Cheng, the Chief Medical Officer and Executive Director of the company.

Summary of our *in vivo* assessment against neuroblastoma and *in vitro* assessment against other cancers are discussed below.

Neuroblastoma *In Vivo* Assessment

Based on the initial 22 day data of a recent study we conducted in a xenograft mouse model of neuroblastoma, SACT-1 was orally administered daily at 60mg/kg in combination of SOC chemotherapy brought a statistically significant tumor shrinkage (unpaired student’s t-test, $p < 0.01$) from Day 15 to Day 22, compared to the control group which received SOC only. Indeed, the combination reduced the tumor size by up to 54.2% in the first 22 days compared with the control (SOC only). SACT-1 appears to be effective in accelerating the effect of the SOC in early time points (from Day 1 - 7 vs control). This further supports our earlier *in vitro* observation that SACT-1 promotes tumor DNA damage and tumor cell death.

Other Cancer Types *In Vitro* Assessment

In addition, SACT-1 was also screened for *in vitro* activity in a panel of over 300 cancer cell lines. Similar to our previous findings against neuroblastoma cell lines, SACT-1 exhibits similar anti-tumor efficacy across one or more other major cancer types, including but not limited to colorectal cancer, leukemia and lymphoma cell lines. As a result, in addition to treating neuroblastoma, SACT-1 may have potential applications in the treatment of other cancers. Based on this discovery, the company plans to carry out further *in vivo* studies to study the efficacy of SACT-1 over other types of cancers to maximize the potential of SACT-1.

About SACT-1

As part of Aptorum Group’s SMART-ACT[®] platform, SACT-1 was discovered from our SMART-ACT[®] platform focused on orphan and unmet diseases. SACT-1 is a repurposed drug targeted for the treatment of neuroblastoma (and potentially other cancer types) especially in combination with SOC chemotherapy. SACT-1’s mechanism has been demonstrated *in vitro* to enhance DNA damage and tumor cell death.

About Aptorum Group Limited

Aptorum Group Limited (Nasdaq: APM, Euronext Paris: APM) is a pharmaceutical company dedicated to developing and commercializing novel therapeutics to tackle unmet medical needs. Aptorum Group is pursuing therapeutic projects in orphan diseases, infectious diseases, metabolic diseases, woman's health and other disease areas.

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

For further general presentation, please visit:

<https://ir.aptorumgroup.com/static-files/ca36cc65-6f23-4105-895e-f5f234ecca1e>

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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 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.

1 <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3668791/#:~:text=Neuroblastoma%20is%20the%20most%20common,dea>

2 <https://www.cancer.org/cancer/neuroblastoma/detection-diagnosis-staging/survival-rates.html>

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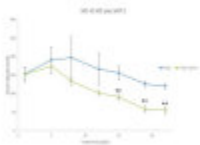
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Source: Aptorum Group

Smart Multimedia Gallery



APTORUM

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**Unpaired student's t-test,
 $p < 0.01$, $n = 8$ (based on initial
22 days period) (Graphic:
Business Wire)

[Logo](#)