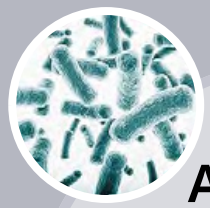
APTORUM GROUP - Novel Therapeutics for Unmet Needs

NASDAQ: APM
Q4 2019 UPDATES

APTORUM

OVERVIEW

- 3 core programs reaching clinical phase in 2020 + menopause supplement commercialisation in Q1 2020
- Targeting US FDA pathways with development sites in North America



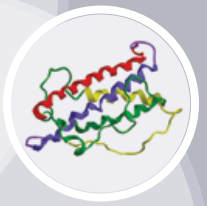
ACTICULE

Anti-virulence approach to bacteria

CLAVES

Pioneering druggable platform in microbiome

APTORUM



SMART-ACT™

Accelerating drug development for orphan diseases via drug repurposing

Additional preclinical programs under development, for details please refer to our website

Current progress of pipeline programs: → Lead Projects → Other Candidates → Projected timeline

Note: all projected timelines refer to the estimated commencement time of the indicated stages

Pillar 1: SMART-ACT™ (SACT series) - Orphan disease drug repurposing platform

Over 7,000 orphan diseases to be screened in the next 5 years

IND 505(b)(2) filing[2]

Program	Indication	Computational Discovery	*In vitro* validation	Existing PhI/II clinical safety data[1]	*In vivo* validation	Bridging studies	PhII / III with limited population[3]
SACT-1	Neuroblastoma				Q4 2019		ready for clinical trial by Q2/Q3 2020
SACT-2	To be disclosed						
SACT-3	To be disclosed						

Pillar 2: Acticule (ALS series) – Infectious diseases[4]

Small molecule, anti-virulence and non-bactericidal approach

IND NDA

Program	Indication	Discovery	Lead Optimization	IND enabling	Phase I	PhII/III based on LPAD pathway[5]
ALS-4	Anti *S. aureus* (incl. MRSA)		+ oral formulation	Q3 2019	Q1/2 2020	
ALS-1	Anti Influenza A			Q4 2020		
ALS-2	Gram+ bacteria					
ALS-3	Gram+ bacteria					

Hybrid study
-volunteers + patients
-initial efficacy readout

Pillar 3: Claves (CLS series) - Microbiota

Large molecule approach. Over 70 targets / indications

IND NDA

Program	Indication	Discovery	Lead Optimization	IND enabling	Phase I	Phase II / III
CLS-1	Obesity		Q4 2019	Q2 2020	Q4 2020	
CLS-2	To be disclosed					
CLS-3	To be disclosed					

Program	Modality	Indication	Formulation	Commercialisation
DOI (NLS-2)[6]	Supplement	Menopausal symptoms		Q1 2020

1. Refers to the drug's existing Phase I/II safety data previously conducted by a third party. Does not refer to clinical trials conducted by Aptorum
2. Subject to FDA's approval on a case-by-case basis, a 505(b)(2) can rely in part on existing information from approved products (such as FDA's previous finding on safety and efficacy) or data in the public domain
3. Subject to the FDA's approval
4. Recent deals in the antibiotic industry (2014-2019): Merck's acquisition of Cubist Pharmaceuticals for US$ 8.4bn; Roivant's licensing of Intron's Phase II asset for US$ 657.5m in milestones
5. ALS-4's eligibility for the LPAD pathway is subject to the FDA's approval. Targeting other indications in Phase II may affect our valuation. QIDP status can be applied once we identify an indication
6. BBC News: "National shortage in hormone replacement therapy adds to the stress of menopause" Aug 2019

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