APTORUM GROUP - Novel Therapeutics for Unmet Needs

NASDAQ:APM Q4 2019 UPDATES

APTORUM

SMART-ACT™ (SACT)

Rare disease universe

No. of Rare Diseases	7,000 & rising
FDA-approved Orphan Drugs	770
Rare Diseases without Treatment	95%
No. of Clinical Trials in Rare Diseases	600
Market Size (US$bn)	US$ 223bn
Total Rare Disease Incidence (US)	10%
Orphan drug sales CAGR (19-24)	12%
Non-orphan drug sales CAGR (19-24)	6%

APTORUM Systematic approach to rare diseases

Non-Orphan Avg. Drug Development Cost	US$ 291m
SMART-ACT™ Avg. Drug Development Cost	US$ 20-40m
SMART-ACT™ Modality	Repurposed small molecule
SACT-1 Indication	Neuroblastoma

ALS-4 (MRSA bacteremia)

ALS-4

Modality	Small molecule
Mechanism	First-in-Class Oral Non-antibiotic
Market Size	US$ 4bn (2025)
MRSA bacteremia incidence (US)	>130,000

Recent deals in anti-infectives

US$ 8.4bn Merck acq. of Cubist Pharm. (2014)

US$ 658m Roivant licensing of Intron's Ph1 asset (2018)

Claves (druggable microbiome platform)

Diseases (70+ indications)	Mkt size
1. Obesity (CLS-1)	US$ 6bn
2. Diabetes	US$ 22bn
3. CV disease	US$ 130bn
4. Renal failure	US$ 93bn
5. Alzheimer's disease	US$ 18bn

Market projection (US)

SACT-1	2018A	2035E
US Total Population (m)	328.1	363.2
Neuroblastoma	2,612	2,891
of which high-risk	1,175	1,301

ALS-4	2018A	2035E
MRSA bacteremia	136,967	172,451
MRSA pneumonia	136,967	172,451
MRSA endocarditis	68,484	86,226
MRSA bone & joint infection	8,950	11,269
Immunocompromised patients	10m+	10m+

CLS-1	2018A	2035E
Obese population (m)	127.3	141

Summary of our assumptions on TAM, price, market share and peak sales

	Stage	Clin. Ph PoS	Incidence (2020,US)	ASP (USD)	Launch Year	2024 M/S	2024 Sales (USDm)	2030 M/S	2030 Sales (USDm)
SACT-1 (Neuroblastoma)	(Repurposed) Ph2/3	24.6%	2,612	204,900	2022	25%	43	50%	111
ALS-4 (MRSA bacteremia)	Preclinical	69.5%	136,967	14,639	2022	25%	284	75%	1,246
CLS-1 (Obesity)	Preclinical	61.1%	127,335,000	513	2024	10%	30	20%	1,030
DOI (Menopause)	Dietary Supplement	n/a	36,520,000	200	2020	1%	52	5%	360

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

Program	Indication	Mechanism	Computational Discovery	*In vitro* validation	Existing PhI/II clinical safety data[1]	IND 505(b)(2) filing[2] *In vivo* validation	Bridging studies	PhII / III with limited population[3]
SACT-1	Neuroblastoma	Drug Repurposing				Q4 2019		ready for clinical trial in 2H 2020
SACT-2	To be disclosed	Drug Repurposing						
SACT-3	To be disclosed	Drug Repurposing						

Program	Indication	Mechanism	Discovery	Lead Optimization	IND enabling	Phase I	Phase II / III
ALS-4	Anti *S. aureus* (incl. MRSA)	Anti-virulence		+ oral formulation	Q3 2019	2H 2020	based on LPAD pathway[4]
CLS-1	Obesity	Druggable Microbiota		Q4 2019	Q2 2020	Q4 2020	

Program	Modality	Indication	Formulation	Commercialisation
DOI (NLS-2)	Dietary Supplement	Menopausal symptoms[5]		Q1 2020

Sources to industry data, market size and financial projections available upon request. For full description of our programs please visit ir.aptorumgroup.com

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. 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
5. BBC News: "National shortage in hormone replacement therapy adds to the stress of menopause" Aug 2019

IR CONTACTS T +852 2117 6611 E investor.relations@aptorumgroup.com W ir.aptorumgroup.com