APTORUM GROUP - Novel Therapeutics for Unmet Needs

NASDAQ: APM Q2 2020 UPDATES

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 case (US)	>130,000

Third-party infectious disease drugs or company-related mergers and acquisitions
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

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	1,969	2,479
MRSA endocarditis	68,484	86,226
MRSA bone & joint infection	8,950	11,269
Immunocompromised patient	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	Case (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
NativusWellTM (Menopause)	Supplement	n/a	36,520,000	200	2020	1%	52	5%	360

Current Progress of pipeline programs: → Lead Projects → Other Candidates → Projected Timelines

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

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

Program	Indication	Mechanism	Computational Discovery	*In vitro* validation	Existing PhI/II clinical safety data[1]	*In vivo* validation	IND preparation & submission	PhII/III with limited population3
SACT-1	Neuroblastoma	Drug Repurposing	→	→	→	→	→	ready for clinical trial in 2H 2020
SACT-2	To be disclosed	Drug Repurposing	→	→				
SACT-3	To be disclosed	Drug Repurposing	→	→				
SACT-COV19	Covid-19	Drug Repurposing	→	→				

Program	Indication	Mechanism	Discovery	Lead Optimization	IND-Enabling	Phase 1	Phase II / III
ALS-4	Anti S. aureus (incl. MRSA)	Anti-virulence	→	→	+ oral formulation →	H2 2020 →	Subject to LPAD pathway[4]

Program	Indication	Mechanism	Discovery	Lead Optimization	IND-Enabling	Phase 1	Phase II / III
CLS-1	Obesity	Druggable Microbiota	→	→	2020 →	2021 →	

Program	Modality	Target Customer	Formulation	Commercialisation
NativusWell™ (NLS-2)	Supplement	Women undergoing menopause to postmenopausal cycle	Launch in Hong Kong in 2020 →	

Source to industry data, market size and financial projections available upon request. For full description of our program 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 trial s 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

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