

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

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)

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	ALS-4	2018A	2035E	CLS-1	2018A	2035E
US Total Population (m)	328.1	363.2	MRSA bacteremia	136,967	172,451	Obese population (m)	127.3	141
Neuroblastoma	2,612	2,891	MRSA pneumonia	1,969	2,479			
of which high-risk	1,175	1,301	MRSA endocarditis	68,484	86,226			
			MRSA bone & joint infection	8,950	11,269			
			Immunocompromised patients	10m+	10m+			

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
NativusWell® (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) filling²

Program	Indication	Mechanism	Computational Discovery	In Vitro Validation	Existing PhIII Clinical Safety Data ¹	In Vivo Validation	IND Preparation & Submission	PhIII with Limited Population ³
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® DOI (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 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

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