AozoraStep

PureTech Health: Perhaps the most overlooked, successful biotech company

Company:	PureTech Health (PRTC LN)	**Market Cap:**	$520mio
Industry:	Biotech	**Net Cash:**	$400mio
Country:	USA, UK, global	**Trial success rate:**	>80% (3 FDA approvals)
Date:	3rd December 2024	**Cash burn incl. R&D:**	<$130mio p.a.
Dividend:	-	**Programmes in trial:**	17
Entry:	$523mio	**Target:**	$800mio (52%)

Why PureTech Health?

- $400mio net cash that lasts at least 3 years
- 3 clinical trial results coming up this month whilst PureTech has a historic clinical trial success rate of >80% and 3 FDA approvals
- $400mio milestone payments from Karuna (KarXT/Cobenfy) to be received + 2% royalty for any annual revenue >$2bn
- Share in founded entity Seaport is already valued at $225mio within one year from $46mio contributions

Risks

- Failure of clinical trials
- Shake up of US health department by RFK

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About PureTech Health

PureTech Health is perhaps the most overlooked, yet very successful biotech company on earth. Over the last 15 years, the company has had a clinical trial success rate of over 80%, received 3 FDA approvals (Cobenfy/KarXT, EndeavorRX/AKL-T01, Plenity/Gelesis100) and its R&D resulted in over 29 therapeutics and therapeutic candidates. The firm has not raised capital since 13 March 2018, and is hence self-funded. This is achieved by bringing in new investors to fund the clinical trials, often early on, which enables PureTech to continuously innovate and not only focus on one product but offer a diversified biotech portfolio. In total, PureTech has a share or 100% ownership in over 8 different drug programmes and over 17 products in clinical trial, plus the recently sold Karuna (KarXT/Cobenfy) to Bristol Myers Squibb for $14bn ($293mio to PureTech). PureTech is at the forefront of oral treatments, oftentimes protein-based that are currently only available via injection or not available at all (Entrega, Vedanta, Seaport, Gallop), genome engineering (VOR) and really combining different treatments and engineering these treatments into a new drug (LYT-100). These 8 programmes are targeting a wide range of diseases, including cancer such as AML, head and neck cancer and MDS (Gallop, VOR), lung diseases (LYT-100), Schizophrenia (Karuna KarXT/Cobenfy), neuroscience (Seaport, Sonde), peptide therapeutics, such as hormones, growth factors, neurotransmitters, ion channel ligands and anti-infectives (Entrega) and gastrointestinal diseases, i.e. bacteria that affect the digestive tract, incl. peanut allergy, as well as infection prevention (Vedanta). The company focuses its therapeutics on diseases that either have a high mortality rate and insufficient existing drugs and/or a very large target group for which no efficient drug exists yet, giving a success of the drugs a potential blockbuster status. The table on the following page summarises PureTech's IP, ownership and near-term milestones. But there is more to explore under the bonnet of PureTech…

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PureTech Health: Current programmes

Drug/ Founded entity	Owner ship	Disease treatment	Clinical trial stage	Next trial publication	Pipeline products	Current valuation (PureTech share $mio)	Comment
LYT-100 (PureTech)	100%	Idiopathic Pulmonary Fibroses (IPF)	2b	Mid-December	1	?	Competitors are Pirfenidone/Esbriet (13.2% market share) (owned by Roche), Nintedanib/OFEV (13.2% market share) (owned by Boehringer Ingelheim), 3/4 of patients cannot handle existing drugs due to side effects, 40% of those taking the existing drugs discontinue due to side effects, life expectancy is 2-5 years (without) and 4.5-7.5 years with the drug. LYT-100 performs better than existing treatments on lower dose potentially omitting side effects. >$4bn market. 1.3mio patients in 16 major markets. IP expiry 2033 and 2044
LYT-200 (Gallop Oncology)	100%	Metastatic solid tumors incl. urothelial & head and neck cancers, AML/MDS	1b	Mid-December	1	?	Received Orphan Drug designation from FDA for treatment of AML (20k annual patients), fast-track designation for head and neck cancer (66k annual patients), >50% of AML patients don't respond to initial treatment or face death, 12.6% 5-year survival rate
Seaport Therapeutics	37%	MDD, anxiety & neuropsychiatric disorder	2b	Mid-December	3	225	PureTech founder has become the CEO, company raised $325mio in a matter of months, Huge addressable market: 280mio with MDD, 120mio MDD with anxiety disorders, 301mio with anxiety disorders worldwide, Glyph platform: SPT-320 bypasses the livre vs. agomelatine, SPT-300 has 9x drug delivery to target vs. orgal allopregnanolone.
Karuna (KarXT/ Cobenfy)	0%	Schizophrenia	FDA approved	-	1	>400	Sold for $14bn to Bristol Myers Squibb (2.3% share or $293mio), >$400mio milestone payments + 2% royalty on >$2bn revenues pa, AbbVie's schizophrenia drug recently failed its stage 2 clinical trial. PureTech invested $18.5mio in Karuna and received $1.1bn to date. 2.8mio patients with schizophrenia (US), 3.2mio patients with Alzheimer (US)
VOR Biopharma	4%	AML, MDS	2a	Mid-December	6	3	42.5k annual AML patients in US, Europe and Japan, median 5yr survival rate with AML patients is <30%, NASDAQ listed entity
Sonde Health	35%	Mental, cognitive and respiratory health	Validation studies	-	2	?	Voice-based AI platform, 17mio individuals are affected by depression in the US
Vedanta Biosciences	36%	Inflammatory, immunology, infection prevention, peanut allergy	3	2025	3	60	500k CDI cases pa in US, 1mio ulcerative colitis & Crohn's disease patients in the US, 4.6mio people with peanut allergy in the US
Entrega	74%	Oral administration of peptide therapeutics	Pre-clinical	2025	1	?	To validate its technology, Entrega generated preclinical proof-of-concept data demonstrating administration of therapeutic peptides into the bloodstream of large animals

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Why is this a turnaround?

The key to understanding PureTech Health is really about knowing how their business model has evolved. PureTech has only invested $18.5mio in KarXT/Cobenfy, but already generated $1.1bn from equity sales and the Bristol Myers Squibb takeover to date. This success has enabled PureTech to keep a higher share of their current innovations without raising external capital. LYT-100 and LYT-200 are both still 100% owned by PureTech, and a success of LYT-100's phase 2b clinical trial would result in high interest from pharmaceuticals to help fund the phase 3 trial. The addressable market for LYT-100 could generate $ billions per annum given that Boerhinger Ingelheim's OFEV generated EUR 3.5bn in annual sales in 2023[1] with 55% market share of ¼ of the total addressable market and expectations that LYT-100 could reach 44% market share[2]. Next, we have Gallop oncology and a success of LYT-200's 1b clinical trial would then be fast-tracked towards phase 2. Whilst LYT-100 has an addressable market of 30-40k patients in the US per annum, LYT-200 could have a combined addressable market of AML and head and neck cancer of nearly 90k patients per year. AML has a particularly high death rate. Finally, there is Seaport Therapeutics, which has raised $325mio in just a matter of months and given the large size of the addressable market with over 300mio people suffering from anxiety disorder, this could become a blockbuster programme rivalling Novartis obesity drug (in the very best-case scenario of course). A successful 2b clinical study could quickly see suitors emerge and the $837mio estimated valuation of Seaport ($225mio PureTech) could multiply. All these three programmes will see their next clinical trial results published this month(!). The upside is quite enormous, while the downside is very low. Why? Because of $400mio net cash, $400mio milestones to be received from KarXT/Cobenfy and a potential 2% royalty on any annual revenue >$2bn for KarXT/Cobenfy. Remember, Bristol Myers Squibb bought KarXT/Cobenfy for $14bn. Therefore, these milestones and royalty income appear more likely than not to materialize since the drug is FDA approved and has no viable competition at this point.

[1] https://annualreport.boehringer-ingelheim.com/2023/download/BOE_AR23_Highlights_2023_EN_safe.pdf
[2] https://puretechhealth.com/images/PRTCCorpPresentation.pdf p. 18

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The financials are strong

As of 30 June 2024, PureTech Health had $400.6mio net cash on its balance sheet. The company is guiding towards a cash burn of around $130mio per annum. However, this cash burn excludes the potential $400mio in milestones from KarXT/Cobenfy (of which $29mio was already received in H2 2024) and 2% royalty on sales >$2bn per annum. Therefore, year-end cash will likely be around $360mio instead of the $330mio guided in H1 2024. If the 80% clinical trial success rate holds, the worst-case outcome would be negative results for LYT-100 and Seaport's SPT-300 with LYT-200 likely playing a less important role given it's a 1b study. However, success in only one of either LYT-100 or Seaport's SPT-300 2b study, should lead to a revaluation of PureTech. With all these biotech companies, it always feels a bit like a flip of a coin, as you never quite know the outcome of the next clinical trial. But given the track record and not one, but three clinical trial results in a single month, there is high potential upside and somewhat protected potential downside amidst the milestones, royalties and $360mio cash balance. I believe PureTech could now be at a pivot point where it is fully self-funded and only needs a partner to market their products, but soon can fund their trials all themselves without giving up equity. The upside is therefore enormous, if the clinical trial results to be published this month turn out to be successful.

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