# Diversifying to Conquer: Fortress Biotech (FBIO) Partnering Expansion Driving Progress in Cancer Immunotherapy and Rare Disease Therapeutics

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**Fortress Biotech** FBIO creates partner companies to manage and optimize cancer immunotherapy and other disease treatment development programs. Its broad portfolio of therapeutic categories includes oncology, rare disorders, gene therapy, neurology, pain management, dermatology and infectious disease.

Fortress believes its unique and progressive structure builds in enhanced financial leverage and flexibility, with the ability to monetize assets more fluidly.

With more than 25 pipeline programs, Fortress' reach has expanded into 11 partner companies. Three have gone public: **Checkpoint Therapeutics** CKPT, **Mustang Bio** MBIO and **Avenue Therapeutics** ATXI, in which Fortress Biotech retains approximately 18% to 23% ownership stakes. In its eight non-public partner companies, Fortress' ownership ranges from approximately 29% to 93%. Additionally, it receives a 4.5% royalty on products sold by partner companies under its standard intercompany arrangements.

The pharmaceutical market, saturated with high-priced drugs, is creating opportunities to enter and expand the sphere, offering equally safe and effective alternatives at increasingly lower prices. This positioning could bring disruptive pricing into the oncology sector, which is known for particularly high-priced drugs.

## Projects in the most Advanced Stages of Development

Cosibelimab, currently in development at Fortress partner company Checkpoint Therapeutics (with technology licensed from Dana Farber Cancer Institute), is a checkpoint inhibitor. One of the conditions it is being developed to treat is squamous cell carcinoma of the skin, the second most deadly form of skin cancer. Current efficacy results for cosibelimab slightly exceed **Merck & Co.'s** MRK 0.07% Keytruda and **Regeneron** REGN and **Sanofi's** SNY Libtayo, two checkpoint inhibitors currently approved for the indication.

Another condition for which a Fortress company has a clinical-stage treatment is Menkes disease: a pediatric genetic disorder that, if untreated, is lethal for most children before age three.  Menkes disease is caused by genetic mutations in copper transporter gene ATP7A, leading to low levels of copper in the body which can cause seizures, development delays, and connective tissue problems. Cyprium Therapeutics is developing CUTX-101, a Menkes disease therapeutic, acquired from the National Institutes of Health (NIH). It is a molecule suitable for restoring copper homeostasis and serum copper levels to normal levels.  Currently, there is no FDA approved therapy for the condition.

In topline efficacy data reported to date, CUTX-101 has been shown to extend life in treated Menkes disease patients, compared to an untreated historical control cohort.  Median survival for patients treated early with CUTX-101 is nearly 15 years, compared to 1.3 years for a historical control cohort. If approved by the FDA, the drug could receive marketing approval by as early as mid-2022.

Checkpoint Therapeutics is approximately 20% Fortress-owned; Cyprium is approximately 70% owned.

Benzinga spoke with Fortress Biotech CEO Lindsay Rosenwald, M.D., who is confident in cosibelimab's ability to position itself well in the market, if approved.

"If cosibelimab is approved, the team at Checkpoint plans to price it aggressively — possibly at one-third less than the going rate of direct competitors — and still maintain pharmaceutical margins," Dr. Rosenwald said. "They expect this to be very appealing to payors and to patients who face sizable coinsurance payments for drugs like Keytruda and Libtayo that cost roughly $150,000 per year — and I think they are right."

Dr. Rosenwald is looking forward to exploring and realizing more of Fortress's objectives coming to fruition.

"We are very excited about the future of Fortress. We have a seasoned group of CEOs running our partner companies, our central staff is motivated and efficient, and we believe we are on the way to proving that the Fortress business model — as unique as it is in the world of biotech — provides the right mix of talent, experience and capital to create and grow companies in a very competitive space."

Later this year, Checkpoint plans to initiate a Phase 3 trial of cosibelimab in non-small cell lung cancer, or NSCLC, the most common form of lung cancer. In 2020, the indication accounted for the bulk of Keytruda's $14 billion in sales.

Following last summer's announcement of positive topline efficacy data, Cyprium plans to initiate a rolling New Drug Application (NDA) submission in the second half of this year – a schedule that could lead to marketing approval in mid-2022.

CUTX-101 may be eligible for a priority review voucher. These transferable vouchers provide for FDA priority review of a separate new drug application and are redeemable or sellable. Each voucher can be worth as much as $100 million or more, based on recorded transactions.

Dr. Rosenwald noted that another partner company, Mustang Bio, also has the potential to receive a priority review voucher based on its program for Bubble Boy disease (XSCID), which is in late-stage development.

A report issued by Cowen & Co. last month predicts Merck will rack up $16.2 billion in 2021 Keytruda sales, with the next three biggest entrants – Opdivo from **BristolMyersSquibb** BMY 0.05%, Tecentriq from **Roche** (OTCMKTS: RHHBY) and Imfinzi from **AstraZeneca** AZN -- booking $8.08 billion, $4.1 billion, and $3.0 billion, respectively, during the same period.

Cowen analysts put the total 2021 checkpoint inhibitor market at $33 billion, growing to just over $50 billion during the next four years, as more and more combination therapies are likely to win approval.

Analysts at Cowen and other firms say the main idea with combination therapies is teaming immunotherapy agents like checkpoint inhibitors with targeted cancer agents (chemotherapy, for example) to increase the durability of response. The concept has created a lot of excitement – notably so far in treating lung, kidney, and liver cancers.

Dr. Rosenwald believes cosibelimab's current safety profile, if sustained in further studies, could put it in a strong position not only as a single agent, but also as an agent of choice in combo therapies.

"So far its safety profile is reading better than Keytruda's, which is the biggest selling drug in the category," he said.

One recent deal that demonstrates the benefits of Fortress Biotech's partner company structure is the transaction between Fortress partner company Caelum Biosciences (approximately 29 percent Fortress owned) and **Alexion** ALXN which could provide Caelum shareholders with up to $500 million in upfront and milestone payments, 43 percent of which would go to Fortress. Another example is the development and asset purchase agreement that Cyprium recently executed for CUTX-101, under which a third-party biopharmaceutical company will provide up to $20 million in development and regulatory cash milestone payments, aggregate sales milestones of up to $255 million and royalties on CUTX-101 net sales ranging from the mid-single digits to the mid-twenties.