IN VIVO
CITELINE COMMERCIAL

07 Feb 2024 | **Analysis**

Unpartnered Assets To Watch In 2024

by Edwin Elmhirst

Licensing serves as a strategic R&D avenue for major developers amid a shrinking M&A market. *In Vivo* has detected five high-value unpartnered assets that might be the focus of dealmaking activity in the coming months.

Licensing as an of external form of R&D and pipeline restocking for larger developers has increased in popularity hand-in-hand with the retraction of the M&A market in recent years. The ability to mitigate risk through milestone payments and other deal structures makes licensing attractive. This is especially true for early stage assets, which is where we are increasingly seeing big pharma place its investments.

However, identifying which assets are worthwhile for investment consideration has always been a challenging process. *In Vivo* has identified five high-value unpartnered assets to watch in 2024. The perceived value of the products is based on the net present value of the drugs according to *Evaluate* data, which is derived from sellside consensus forecasts. To qualify for inclusion the assets needed to be wholly owned by their developers, outside of any minor region deals, and are unlikely to be launched by that company.

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Honorable mentions

CB010 - Caribou Biosciences

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Although *Caribou Biosciences, Inc.*'s CAR T-cell therapy CB010 is still at a very early stage, the company has stated they have made positive steps with the US Food and Drug Administration to get a Phase III trial in second-line diffuse large B-cell lymphoma (DLBCL) running by end of 2024. Even so, those estimating a launch as early as 2025 may be being ambitious – with more conservative broker estimates being that the drug could be ready for market in 2027/2028.

The allogenic cell therapy space is a hotly contested area where Caribou hopes to differentiate CB010 with its PD-1 knockout strategy. Over the course of 2023 Caribou delivered data addressing durability concerns, a common problem in allogenic Car-T projects, and maintained a strong safety profile. Unlike many other players in this space though, the company has made no mention of exploring autoimmune indications, yet.

In addition to the usual development risks, CB010 also faces a new threat from a recent development pivot from rival company Allogene. In January 2024, Allogene decided to move directly into first-line trials in DLBCL. Whilst this move is risky for Allogene's Cema-cel it is still on a similar timeline to CB010 and success could prove very damaging to CB010's market hopes.

Caribou has already attracted the interest of larger entities, managing to secure a $25m equity investment from Pfizer in July 2023.

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Pemvidutide – Altimmune

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Altimmune Inc. is actively seeking partnerships for pemvidutide in both Obesity and nonalcoholic steatohepatitis (NASH) – ideally to find the same licensee for both. The company is looking to get a partner on board by the time it starts the first Phase III trial for pemvidutide – which will be in obesity.

Altimmune has also featured on many speculative biopharma M&A target lists, especially as interest in the therapeutic obesity market hots up. A position only reinforced by Roche's $2.7bn upfront takeout of Carmot, a fellow GLP-1 developer, in December 2023.

Altimmune posted new and improved data in December for the Phase II MOMENTUM trial in obesity. The updated results showed increased efficacy and lower dropouts. SC149439 However, despite the improvement on previous data releases, side-effect related discontinuations for pemvidutide will have to be reduced in Phase III to keep step with marketed competitors.

While the markets for NASH and obesity are vast, the challenge for Altimmune and its future partner will be in trying to wrestle market share from well-established rivals, such as Novo Nordisk's Wegovy (semaglutide) and Eli Lilly's Zepbound (tirzepatide).

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GTX-102 – Ultragenyx

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Ultragenyx Pharmaceutical Inc.'s GTX-102 has been left the leader in the Angelman syndrome space after Roche shelved research for rugonersen in June 2023. Unless Roche finds a partner to take over the development of rugonersen, the closest remaining competitor in this indication is

Ionis's ION582. It seems likely that it will be up to GTX-102 to set the bar for performance of the other UBE3A antisenses in development.

The first half of 2024 should see more Phase I/II study data being released for GTX-102, including from an expansion cohort. Good results in this 20 patient group could pave the way for a Phase III trial getting underway by the start of 2025. Development of the antisense oligonucleiotide has been slower than initially hoped with setbacks in late 2021. The program was paused after patients experienced severe lower extremity weakness and dosing had to be amended. *Evaluate* predicts a potential launch date in 2026, however, this is likely a bullish estimate.

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XEN1101 – Xenon Pharmaceuticals

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In recent years there has been increased interest in late-stage, de-risked CNS assets as both takeout and licensing targets. *Xenon Pharmaceuticals, Inc.*'s epilepsy drug XEN1101, as with many other assets in the same class, has the potential to also launch in major depressive disorder. The company plans to start a Phase III trial for this indication in 2024. Both epilepsy and MDD are very large markets, currently served by relatively old drugs.

Xenon has a reasonable cash runway for clinical activities. The company has also indicated a preference to commercialize the program itself, but anti-seizure medications can face slow launches. Some brokers have highlighted concerns that a solo launch by Xenon may not be feasible without additional capital.

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Paltusotine - Crinetics Pharmaceuticals

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Crinetics Pharmaceuticals, Inc.'s paltusotine is poised to potentially become the standard of care in acromegaly if it can continue to deliver similar results to those seen in the Phase III PATHFINDR-1 trial, which read out in September 2023. SC149029 The somatostatin receptor type 2 is looking to gain access to the complete spectrum of patients in acromegaly – recruiting both treatment naïve and wash-out patients for PATHFINDR-2.

In addition to its hopes in acromegaly, paltusotine is also in Phase II for carcinoid syndrome. Initial trial data for this indication have also been positive and full data are expected in the first half of 2024.

Both the acromegaly and carcinoid syndrome markets are dominated by injectable somatostatin receptors ligands. An oral treatment option may be welcomed by patients but there are concerns about diminished compliance for what is often a well-controlled condition.

Crinetic's has yet to go into detail about any go-to-market plan for paltusotine and the endocrinology market can be tricky – especially in terms of access. However, there is already a partnership in place for the Japanese market which was inked in February 2022 with Sanwa Kagaku Kenkyusho .

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Honorable mentions

In addition to the above assets, *In Vivo's* analysis of valuable R&D drugs also revealed several companies which, although planning to commercialize in US themselves, are seeking specific regional partners.

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