# New Model for Sharing Drug Development Risk: Offloading All of It to Development Partners

**By Chris Morrison**

Bosulif, Pfizer's first ever blood cancer treatment, was initially approved in 2012 as a second-linae therapy. When the Food and Drug Administration approved it in December 2017 to treat patients with a form of newly-diagnosed chronic myelogenous leukemia, it significantly expanded the drug's label in the U.S. The approval was also a validation of Pfizer's decision to entirely outsource the Phase 3 study – it's financing, execution, and regulatory interactions – that led to the new indication.

For pharmaceutical companies with finite R&D budgets, finding partners to help shoulder the financial burden of expensive clinical trials is well trodden risk-sharing path. But in recent years a new model for sharing drug development risk has emerged, one where a development partner will take on the entirety of the costs associated with a particular clinical program in exchange for being rewarded if – and only if – that program succeeds with regulators.

The Bosulif study was conducted and financed by London-based Avillion, a company founded in 2012 to finance and run late-stage clinical development and regulatory approval for large biopharmaceutical company assets that might fall outside the scope of a company's R&D budget or resources.

Essentially, Avillion takes the clinical and regulatory risk of a partner's development program – "100% of it," says Jeynes-Ellis – and gets rewarded upon regulatory approval. This first success validates its business model and may indeed spark others to adopt it; FDA granted Bosulif a coveted Priority Review and the drug received an accelerated approval in its new indication. In February 2018, a European Medicines Agency (EMA) committee recommended that the EMA also approve the drug as a first-line treatment. Pfizer retains global rights to market the drug and Avillion will receive unspecified milestone payments.

Dr. Allison Jeynes-Ellis, CEO, Avillion

Bosulif was a good fit given the company's depth of expertise in oncology and immunology, explains Jeynes-Ellis, but the company isn't stuck in those particular therapeutic areas. "We'll bring in experts to fit pharma partners' needs," she explains. For a recently signed deal with Pearl Therapeutics (a division of AstraZeneca), the drug developer is adding pulmonologists to its team, for example. That deal, around Pearl's PT027 asthma program (a fixed-dose combination of the inhaled corticosteroid budesonide and the short-acting beta-2 agonist albuterol), was signed in March 2018 and followed a March 2017 deal with Merck, the German pharmaceutical company, to develop the anti-IL-17 A/F nanobody M1095 for psoriasis.

Avillion's founding investors are Clarus Ventures and Abingworth, which have prior experience with this late-stage development model through SFJ Pharma, a company set up in 2009 to

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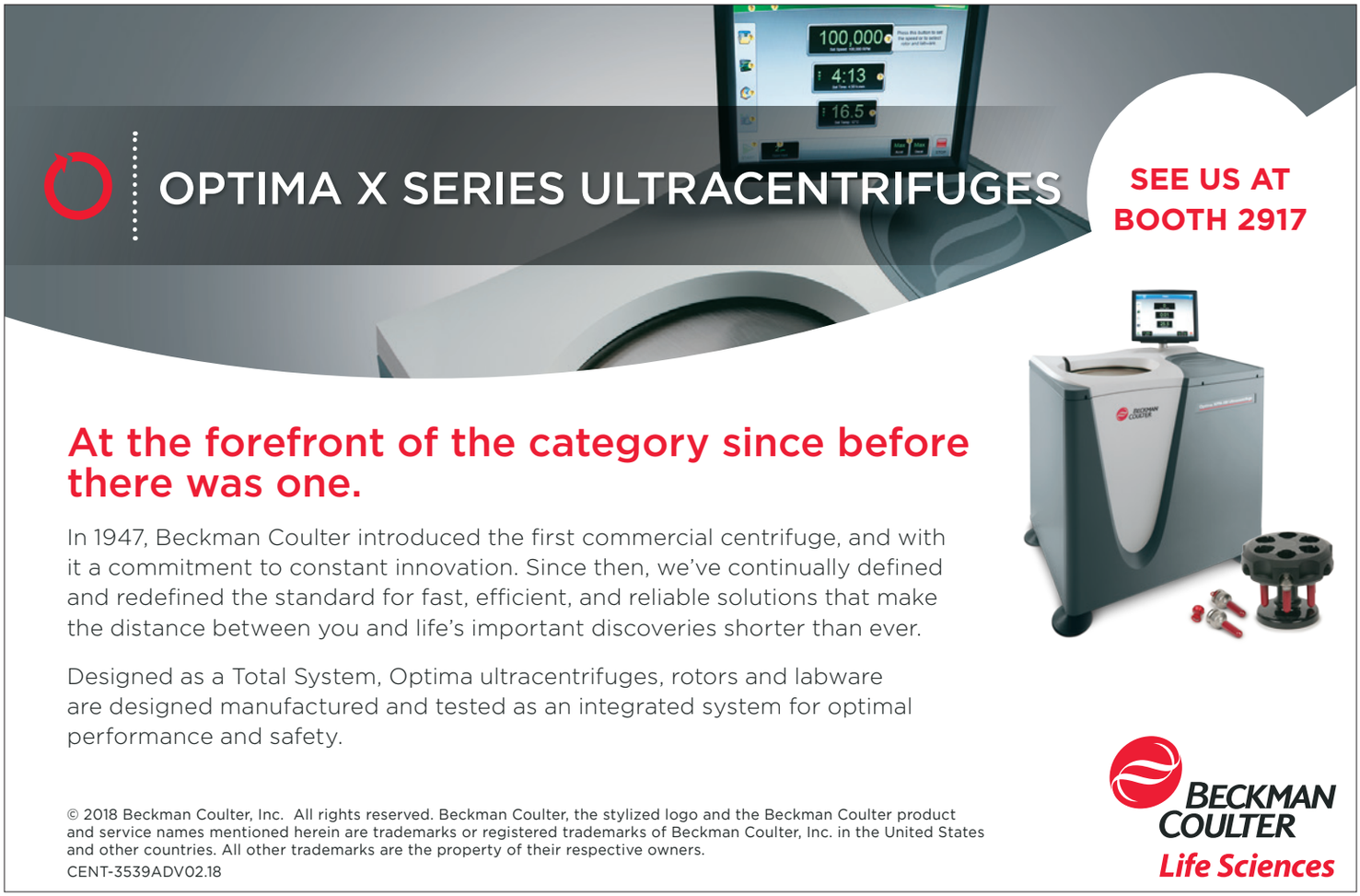

## BIO 25 YEARS OF BIO INNOVATION Trailblazers

*While most people involved with biotechnology companies felt it was important to have solid, strong representation in Washington, and we had seen the difficulties of two different groups representing biotechnology, the overwhelming thing I remember was the excitement for the potential of the technology, and the promise that the technology could be translated into unimaginably beneficial new therapies for patients. That excitement probably seemed like a crazy dream to most people at the time. But the dream came true.*

**Tom Wiggans, Chairman and Chief Executive Officer, Dermira, Inc. Tom served approximately 6 years on the BIO board from the late 90's through the early 2000's.**

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similarly finance development and registration of pharmaceutical partners' projects (Royalty Pharma joined as an Avillion investor in May 2014, and SFJ has additional institutional investors as well). SFJ has also recently found success partnering with Pfizer around the Mylotarg, an antibody-drug conjugate approved in the US in September 2017 and recommended for EU approval in April 2018 in a type of acute myeloid leukemia.

These models aren't without blemish, however. In April 2018 Pfizer said a Phase 3 trial of its cancer drug Inlyta in renal cell carcinoma was halted by a monitoring board when it became clear the trial would fail. That trial was also backed by SFJ. Still, Jeynes-Ellis points to increasing interest from potential collaborators and development specialist's increasing ability to be choosy.

"Before we signed the deal with Pfizer, I had personally done due diligence on a hundred assets," says Jeynes-Ellis, who until February 2014 was the company's chief medical officer. But that ratio has "totally and dramatically" changed, she says. "Companies are proactively engaging with us," she says, sometimes around their most important assets. To reflect the needs of pharmaceutical partners the company has had to tweak its business model. For example, only a few years ago, Avillion would only consider deals around pure Phase 3 assets. Its deal with Merck moves it earlier, to riskier Phase 2 clinical development.

In March 2018, that program achieved its first operational milestone when FDA accepted its investigational new drug application for M1095. Moving earlier in development and taking on projects, like Pearl's PT027, outside of oncology and immunology is reshaping the company. Jeynes-Ellis expects it to double in size over the next six months, as it builds out its asthma capabilities and reinforces its US presence.

One area Avillion won't branch into is commercialization, a key reason the company is only likely to partner with companies that already have a large commercial presence and around assets in partners core therapeutic areas – assets they'll drive forward commercially only if its development partner achieves clinical and regulatory success.