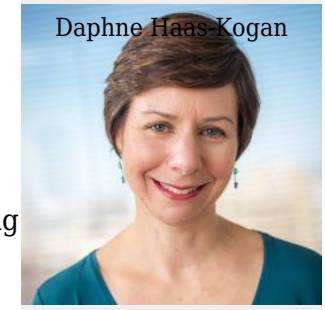
ENDPOINTS NEWS

A new biotech from a longtime developer wants to bring the targeted cancer revolution to the kids left behind

by Jason Mast on May 21st, 2020

Daphne Haas-Kogan was treating and studying children's brain tumors at the University of California San-Francisco, when she got a call that shook her. The pharma company whose drug she had been prepping for a trial had decided, despite all preclinical evidence, to not run any trials on kids, only adults. Haas-Kogan's patients would not get the therapy.

"Ultimately the company had to make a decision for what trials they would support," Haas-Kogan said. "I can still recall my blood pressure rising as I found out."


Daphne Haas-Kogan

The last two decades have seen a revolution in treatments for cancer, bringing targeted therapies that have extended some patients' lives by years or

decades. But much of that revolution, experts say, has skipped childhood cancer. For biotech and Big Pharma, these patients represent a much smaller patient pool, making it more difficult to run trials and giving less market incentive to try them. They come with greater societal concerns on safety, and for years the biology was less understood.

So the treatments for many remain different formulations of the same interventions that doctors have used for decades: radiation and chemotherapy, both of which often come with deep long term side effects. “The world is not that different today” than it was 20 years ago, said Vickie Buenger, president of the Coalition Against Childhood Cancer and a researcher who studies the business of biotech.

A new biotech is trying to change that, one drug at a time. Founded by an extensively-pedigreed cancer drug developer and a young venture capitalist, the company, called Day One, has raised $60 million from Atlas, Canaan, and others behind a vision of finding shelved and other drugs with promise for kids, licensing and bringing them forward. They’re starting with TAK-580, an old Takeda therapy they’re now testing in children with gliomas.

“Everybody in drug development can get behind helping kids with cancer – everybody,” CEO Julie Grant told *Endpoints News*. “We’ve just really focused on how to align incentives and how to align behavior and opportunities so that kids can not only get access to any product but get access to the best product and as quickly as possible.”


Sam Blackman

The idea for the company emerged a couple years ago when Sam Blackman went to Canaan’s offices in San Francisco to pitch Grant on his newest cancer company. Blackman had worked as a scientist and executive at Merck, GlaxoSmithKline, Seattle Genetics, Juno Therapeutics and Silverback Therapeutics, but he began his career as a pediatric oncologist, focused on brain tumors, at Dana Farber and Boston Children’s Hospital. For years, amid his day-to-day research, he had been trying to figure out how to get companies to invest in kids. He was even tasked to lead a working group on “Setting Up New Business Models and Ways to Invest in Paediatric Oncology Research and Drug Development.” Accelerate, it was called.

So after the pitch, for his new, general oncology company, Grant told Blackman about an idea she had been working on for a biotech that would focus exclusively on kids.

”I had been wondering, ‘Is there some way to build a company that would be focused on pediatric oncology?’” Blackman told Endpoints. “But I’m not a company builder, I’m really just a drug developer.”


Delphine Heenen

The obstacles to such an idea, though, were myriad. In addition to the fact that there were fewer cancers in kids, the ones that appeared were also different cancers. “Kids do not get ovarian or breast cancer,” explains Delphine Heenen, who chaired the working group alongside Blackman but is not directly involved in Day One.

Companies have also been worried about causing toxicities in children — a concern that comes from a good place but has often stunted development, said Haas-Kogan. Often, the potential harm of disease progression, outweighs the potential harm of a drug. “The anxiety — although I understand it — I think its misplaced,” she said. “Children, I think, may very well have more to gain and the rarity of pediatric tumors leaves them rather limited access to cutting edge treatments.”

Laws in the US and Europe require companies to test adult cancer drugs in kids with similar malignancies, Heenen said, but that largely only applies to approved drugs, leaving out the vast majority of drugs that fail in adults but could be useful in kids.

Haas-Kogan eventually convinced the company to start a trial at UCSF on the drug they initially denied — it’s still under investigation — but it took a concerted advocacy effort. Overall, only 10 new drugs have been approved specifically for pediatric tumors since 1990 and it takes an average of 6.5 years for a drug approved in adults to reach kids, according to numbers Day One cited.

So Blackman was surprised when Grant broached the idea, but excited. “It was honestly the first time a venture capitalist has ever asked me about pediatric oncology,” Blackman said. Grant said she had been unconvincd when Susan Weiner, a cancer research advocate, and Peter Adamson, a drug developer and the chair of the Children’s Oncology Group, brought the idea to her. “The idea really came from the children’s oncology advocacy community,” Grant said. “We built this as a service to them.”

Over meetings at New York and at AACR, though, the four hashed out a plan. “They convinced me that things had changed,” Grant said of Eisner and Adamson.

Blackman and Grant say that we now understand children’s tumors better. With biotech’s experience in rare diseases, we now know how to better run trials on small patient populations well enough. Additionally, they said, the pivot to immuno-oncology — a field that isn’t really germane to childhood tumors — has put other targeted treatments on the shelf, making Pharma inclined to license them out to a company that would test it and give it a shot to get to approval, both in kids and adults.

That will begin with TAK-580, now renamed DAY-101. The drug, originally developed by Sunesis, is a pan-RAF inhibitor, blocking mutations that drive cancer in both childhood and adult gliomas. Haas-Kogan, now at Dana Farber, is co-leading the investigation. The company is looking to license in new drugs and test them in the same way. More than bring any particular compound to FDA approval, Blackman and Grant are hoping to prove to the rest of the industry that what they're doing is possible, that you can make drugs for kids and make money.

"If we're right, we think people are going to copy us," Grant said. "And we are ok with that. If the best thing that comes out of this is that prioritizing children with cancer is a really smart idea, we have done a lot of good."

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