

Emerging Drug Developer: TransMolecular

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TransMolecular stays focused on a unique cancer program

There's one key point that the CEO of Cambridge, MA-based TransMolecular wants everyone to know up front.

"We are not using scorpion venom," says Michael Egan "It is one peptide in 40 in the mix of the venom."

For a biotech audience, that won't be too startling a revelation. But you'd be surprised how often it comes up, he says, in the stories that cover the company's efforts.

TransMolecular has a very narrow focus that covers a broad range of cancer therapies. Starting with research from the University of Alabama, the developer has been advancing TM601, which has the uncommon ability to bind and intrude into a tumor cell. And the implications that they had a new therapy that could act as an independent delivery agent, or perhaps even work as a therapeutic on its own, has consumed the company.

So TM601 became the company's sole concern.

"We had some key questions in the development process that we wanted to answer on this molecule," says Egan, "as opposed to taking on two or three oncology projects. The question was when it bound, what happened.

"Could we give it intravenously? What is it binding to? It sounds trivial, but it turns out to be quite a question," says the CEO. "We identified the binding site, but we haven't published it yet. It's a receptor expressed on the surface of tumor cells."

Then the company asked if TM601 had therapeutic activity by itself.

"We hunted around to see if there was inherent activity. And when you get to higher doses it

inhibited blood vessels in the region of the tumor."

In the first clinical trials, glioma patients were given a dose through their surgical cavity.

"When original delivery started, it was local delivery in recurrent glioma patients who had the surgery. Then we completed the Phase II and we have early indication of increased life span. And now we're talking to the FDA about Phase III.

"Medically," adds Egan, "the key was could you give it intravenously?"

The company organized several Phase I trials where they delivered the therapy by IV and saw the kind of uptake in the tumor they were looking for. Now they've launched a Phase I/II intravenous trial that scales up to a mid-stage study. Researchers will recruit more than 50 patients for this study.

So far, TransMolecular has raised about \$42 million, with Tullis-Dickerson TVM Capital and Easton Capital on its list of investors. The company moved from Birmingham to Cambridge MA and the developer now has a staff of 21.

To stay on track, the CEO is putting together another venture round before the end of the year. But don't ask him how much he plans to raise.

"In this environment, you've got to be kidding me," he says with a laugh.

He's equally quick to chuckle at the idea of planning for an IPO anytime soon.

"That discussion is a nonstarter," he says with another laugh.

Partnerships, however, are definitely on the table.

"A multi-partnership company is a possibility," says Egan. "If someone is interested in acquiring the asset, I think then it becomes more of an M&A.

"We're talking to folks who have a molecule they need to deliver specifically; that's on a couple of fronts," he adds. "We're in a process now with the objective of having partnerships in place in the next 12 months.

"In all honesty, I think our ability to take things into Phase III is pretty limited. You can do I and II in a very focused way, managing your expenses. In a Phase III scenario, that changes completely."

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