

**INSIDE BUSINESS | A WEEKLY CONVERSATION WITH SAN DIEGO  
NEWSMAKERS**

## **Biotech company has survival skills**

### **Ambit Biosciences overcomes challenges, now has new partner**

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David Brooks / Union-Tribune

Scott Salka is chief executive of Ambit Biosciences, which provides services to drug developers and is working on drugs of its own.

It's been a decade of ups and downs for Chief Executive Scott Salka of San Diego's Ambit Biosciences.

The company started in 2000, in the euphoric days of the biotech stock boom. Initially it was a so-called tool company, a popular business model at the time, that would provide services to other companies developing drugs.

But the biotech bubble popped, tool companies fell out of favor, and Ambit cast about for several years for a workable strategy. A low point came in 2004, when the company couldn't come up with rent money and was evicted from its offices.

Now Salka believes he has the privately held company on track. While continuing the services side of the business, the 85-person company has just started a registration trial that could support government approval of its first drug, a treatment for leukemia.

On Friday, the company announced a development and commercialization partnership for the leukemia drug and other, earlier-stage drugs with the Japanese company Astellas Pharma. The deal includes a \$40 million upfront payment, with potential milestone payments and royalties to follow.

Salka spoke Friday about the company and its outlook:

**QUESTION:** For those who've never heard of Ambit, can you give a quick summary of what the company does?

**ANSWER:** We started out as a technology platform company with the aim of building tools that would allow our partners to advance drugs and put them in the clinic. In 2004, we decided that we were going to use our own tool. So in addition to continuing to provide it to partners to help them develop the best drugs possible, we were going to use it ourselves to try to advance some of our own drugs, homemade drugs, into the clinic. It's worked out really well for us.

We moved our first program into the clinic in 2007. We announced just last week that we're starting a registration study with that drug. We started enrolling patients in November. So if all goes well with that registration study, we could see first commercial sales in late 2011, early 2012. That's our first drug. We have another drug that's in (earlier-stage) studies. It's also for cancer. And we have a pipeline of drugs behind that. So we're a local drug development and discovery company that uses our own proprietary tool that's helped a lot of other companies, and now we're helping ourselves.

**QUESTION:** So you're still using the tool in partnerships with other companies. How does that side of the business work?

**ANSWER:** Some are partners, and some are what I would call customers. We formed a true partnership with a company called Cephalon, which is a very successful biotech company in the Philadelphia area. They have a couple of drugs on the market and have over

\$1 billion of revenue a year. We formed that partnership in 2006 and, fingers crossed, the first drug from that partnership will enter the clinic in late 2010, early 2011. We'll get milestones and royalties. The rest of what we do there, I would really characterize those as customers of our technology platform. Those people send us molecules that they are interested in potentially developing as drugs, and we will provide information back about those molecules and how those molecules interact with an important set of drug targets.

**QUESTION:** Does this bring in significant revenue?

**ANSWER:** It has. We started offering this on a fee-for-service basis in 2004, and we doubled revenue for the first four years. Revenue still grows at a very nice rate. As an example, we did over \$20 million in revenue with that part of the business last year. It's significant for us. It gives us access to customers, ideas for what to do with our own drugs. We have also used it as a kind of trading chip to get not just cash, but also access to chemistry, intellectual property that we can use to make sure that drugs we're advancing have adequate patent protection. We used the technology as a trading chip to in-license a drug from Bristol-Myers Squibb in 2007.

**QUESTION:** For a lot of biotech companies, the big issue in recent years has been raising money. Does that revenue insulate you from that at all?

**ANSWER:** I wish it were insulation in the way I think you mean, like big insulation or a big parka. It's more like a sweater. It is very expensive to discover and develop drugs. So the cash flow is meaningful to us, from the technology platform, but it doesn't pay the full freight for all the work we do on the discovery and development side. As an example, our spend next year — because we have a couple of clinical trials going on and we have other compounds that are advancing toward clinical trials — our gross spend next year will be on the order of \$40 million.

**QUESTION:** You have the lead drug well along in clinical trials. Can you tell me a little bit about the effectiveness you've seen so far?

**ANSWER:** We issued a press release last week talking about our potential registration study. That will be in 180 patients with AML, which is a devastating form of leukemia. Patients with the particular flavor or variant of the disease that we're targeting for our pivotal study, well over 75 percent of these patients die in the first year that they're diagnosed. So it's acute myeloid leukemia with an emphasis on acute. It comes on very fast.

We released the data from our Phase I study, and we saw unprecedented single-agent efficacy with a drug that's not a chemotherapy. Most people know chemotherapies as poisons, basically. You dose a patient, and you try to kill the cancer before you kill the patient. This is not a chemotherapy. This is a very well-tolerated, very molecularly targeted agent that leaves the patient feeling fine. The patients stay at home. They take the drug once a day. It's a little bit of powder dissolved in water.

We saw great results in a patient population that chemotherapy has not been successful in. If we can recapitulate that response in this potential registration study, I think we have a really good shot at getting accelerated approval for the drug.

**QUESTION:** When you say great response, are you looking at tumors or at how long the patients survive?

**ANSWER:** We were specifically looking at the tumor. This is a liquid tumor, so it's in the blood and in the marrow. So we're looking at kind of the tumor burden, and we're looking at reducing the tumor burden. As a secondary endpoint, we measured duration of that effect. In our registration study, we'll also be looking at response, but an important secondary endpoint will be duration of that response.

**QUESTION:** You announced another partnership Friday morning. What's that all about?

**ANSWER:** Yes, it's a really important partnership for us. It's our first clinical-stage partnership. We started talking to potential partners earlier this year, and we had deep interest. We ended up doing the deal with Astellas, which is a major Japanese pharmaceutical company, one of the largest pharmaceutical companies in the world. Part of their strategic plan over the next decade is to become one of the major oncology players in the world. So they have done a handful of key collaborations and acquisitions over the last couple of years.

Their CEO has a quote in the press release that talks about how this is one of the key pieces in their strategy. We really like that, as a small company. We plan to co-develop this drug, and co-market in the U.S. with them.

So this marks a watershed moment for the company that shows that what we've been working on is working.

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