

Baby Bio: Big Pharma's Best Weapon in the War on Cancer July 28, 2016

While the war on cancer rages on, some of the biggest therapeutic developments are being made by small pharmaceutical companies, not Big Pharma. Many of these innovative companies may be takeover targets as the big brands hedge their bets and sit out on bench research until they see promise in later stage testing.

At the annual American Society of Clinical Oncology (ASCO) gathering in June, industry experts gathered to discuss a spectrum of cancer-related topics, including specific strains of the disease and breakthrough treatments.

Few outside the pharmaceutical world are likely familiar with a company called Ganymed, a German biotech firm, but it made news at ASCO when releasing data showing its new drug added a five-month survival benefit to chemotherapy patients with gastric cancer.

Big results also came from Ariad Pharmaceuticals, which presented research that its lung cancer treatment may help those no longer seeing benefits from Pfizer's Xalkori and that its drug, brigatinib, may have longer-lasting effects than other treatments currently on the market.

Ariad's stock rose on Thursday thanks to strong earnings and on expectations for its pipeline of new drugs.

The breakthroughs being made by small players in the industry are making a difference. Cancer patients have come a long way on the overall survival front. According to an update by the American Association for Cancer Research's Cancer Progress Report, since 1971 the percentage of the population living with, through or beyond a cancer diagnosis has more than tripled. In the mid-1970s, a person diagnosed with cancer had a five-year survival rate of 49 percent, contrasting dramatically to the 68 percent five-year survival rate in 2010.

Even so, despite extended life expectancy and improved treatments, the National Institutes for Health (NIH) estimates that 8.9 million people die worldwide each year – that's one every 3.5 seconds – from some form of cancer. And here in the United States, the NIH estimates that spending on cancer drugs could reach \$156 billion by 2020.

One way that scientists continue to tackle the disease is through new innovations and therapies that treat the patient as well as the disease. From Aug. 1, 2014 to July 31, 2015, the Food and Drug Administration (FDA) approved nine anti-cancer drugs, one cancer prevention vaccine and one cancer screening test, according to the NIH. To an outsider, these numbers might seem small, but they are enormous, and they only tell part of the story.

According to Charles Travers, a portfolio manager with Motley Fool Asset Management, it can take up to a decade for a treatment to go from inception to successfully crossing the FDA approval finish line. Moreover, nine out of 10 new drugs that begin the testing process won't see the light of day simply because they fail a portion of the three-phase, multi-stage process they must clear before making it to market.

Travers said the smaller, upstart companies that are innovating are best positioned when tackling diseases because they aren't in a crowded field. Over the past decade or so, that has often tended to be cancer drugs, which were developed out of smaller companies and then acquired by big name biotech or pharma companies.

The advent of smaller, more niche-specific therapies proves to be no exception. "These therapies are too small for a big company like a Pfizer or Merck to go after," he said. "A market that will do \$300-\$500 million in sales is great for a small company, but it's not doing much for a big company. The small company has these markets completely to itself."

It's not surprising that many of the largest pharmaceutical companies look to acquire the innovations of their smaller rivals. A study by Bain & Company found that the pharmaceutical companies delivering the most shareholder return made strategic acquisitions to supplement their own declining research and development budgets (R&D).

Greg Hagood, senior managing director of SOLIC Capital, said that about 15 years ago, bigger companies conducted extensive R&D. Over time, however, these major players have cut back on their R&D budgets, opting instead to let the smaller companies and medical campuses take the wheel on that front. Big pharma is content to pay a premium on the backend once a treatment becomes partially or fully approved. "They've been cutting back on R&D," he said. "They'd rather put advertising money behind the product."

One recent example of a large company merging with an innovative upstart came in 2013 when Amgen bought Onyx Pharmaceuticals to the tune of \$10 billion, a move that Travers considers to be one of the most high profile examples in the cancer treatment space in recent years. "Big, established biotech and drug companies that do have big sales forces and portfolios find it hard to develop drugs, so they buy up small companies with innovative, interesting products instead," he said.

At the end of May came another major acquisition driven by a potential leukemia treatment when Celator was purchased by Jazz Pharmaceuticals. Jazz is paying \$1.5 billion, which priced shares at \$30.25, a 72 percent premium above the previous day's close and more than six times revenue estimates. Celator had been valued at under \$50 million earlier in 2016. Big Pharma is willing to pay huge premiums over revenue estimates, as has also been true during the ongoing boardroom battle between Medivation and Sanofi. And analysts believe that fight could stretch into next year with the ultimate buyer paying nearly 50 percent more than the current offer.

Large firms were likely kept busy at ASCO scouting their smaller rivals. Following the announcement of Ariad's data at ASCO, an analyst at RBC Capital Markets quickly noted the company could become an acquisition target. Similar takeover expectations are sure to arise for the next small company to release positive research on its drug pipeline.

Moreover, according to Hagood, innovation doesn't always necessarily mean getting new drugs on the market. Rather, there are times when a patient simply needs a custom-made pill or therapy with "less of this and more of that" in it. "[This is] evolving rapidly now, and cancer programs around the country are testing and trying different combinations of drugs to see if they stop cancer or tumors," he said. "It's moving so fast and there's not a lot of protocol in place now to get some of these treatments qualified."

An area that Hagood and Travers agree has become ripe for growth is in the personalization of cancer treatments. Gone are the days when all patients diagnosed with a particular form of cancer are given the exact same treatment. Gone also is the two-step process of removing tumors and then implementing aggressive chemotherapy post haste. Instead, using a combination of technology and gene therapy, there is an opportunity to classify and treat individual tumors based on their severity.

"One benefit of all genetic knowledge that has come out has led to the realization that no one with breast or prostate cancer has the same disease," said Travers. "There is an ability to tailor treatments accordingly, based on the genetic profile of a tumor," which can mean sidestepping aggressive and costly treatments like chemotherapy entirely for certain patients.

Hagood believes one of the greatest equalizers to come along in a long time is the Internet, which patients can employ to help suss out facts, options and innovations with the click of a mouse. Digitized medical records and widespread data help fuel this trend, while financial investment by major digital players like Google and IBM's Watson cement the notion that modern medicine is ready to come of age online.

In a sharing society, "The holy grail is to get people to start sharing data off of their Apple iPhone and wearables like FitBit about their lifestyle and healthcare stats," Hagood said. "The flip side is that there is a lot of great research going on around the country that we're seeing now. There are genetically based therapies to find the cause of what's causing the tumor to grow instead of removing the tumor."

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