# Taking Aim at Alexion, Ra Pharma Heads to IPO Queue

**Ben Fidler**

October 3rd, 2016

- **@benthefidler**
- **@xconomy**
- **Like Us**

**Xconomy Boston** —

Paroxysmal nocturnal hemoglobinuria may be an ultra-rare blood disease, but Alexion Pharmaceuticals makes over $2 billion a year by selling the only approved drug for it, eculizumab (Soliris). Ra Pharmaceuticals is one of those developing an alternative, and the Cambridge, MA, company is looking to Wall Street to help fund the clinical work.

Ra filed **for an IPO late Friday**, aiming to fund the clinical development of an experimental drug called RA101495. Ra plans to start Phase 2 trials of the drug early next year and release data in the latter half of 2017; the company hopes the trial will build its case that the drug is a threat to Alexion's (NASDAQ: **ALXN**) eculizumab.

Ra's RA101495 is subcutaneous injection for people with PNH, a disease in which the complement system—a part of the immune system—destroys red blood cells, causing a host of problems like blood clots and organ failure. Alexion's eculizumab—which costs more than $500,000 per patient for a year of treatment—is the only approved drug for PNH. It doesn't cure the disease, but it stops the complement system from attacking red blood cells, which in turn lowers the risk of blood clots and other problems associated with PNH. Eculizumab, however, has to be infused bi-weekly at a clinic, and as Ra says in its prospectus, sometimes patients' symptoms recur anyway. Ra estimates that there are about 16,000 patients with PNH worldwide.

Ra is one of several companies trying to develop new drugs for PNH, including Novartis, Amyndas Pharmaceuticals, Apellis Pharmaceuticals, Akari Pharmaceuticals, and Alnylam Pharmaceuticals. Alexion is also developing **a longer-lasting version of eculizumab**.

Like eculizumab, Ra's drug blocks a protein in the complement system called complement component 5. But RA101495 is a peptide drug, not an antibody, and it's taken via an injection just under the skin. Ra believes that a drug like this, self-administered by patients at home on a more frequent basis (either daily or weekly), could be more convenient for patients and help them better control their PNH. The drug is the first product from a platform Ra is trying to show **can combine some of the best properties of antibodies and small molecule drugs**.

Ra is also developing RA101495 for two other autoimmune diseases, lupus nephritis and refractory generalized myasthenia gravis, which causes progressive muscle weakness. Phase 2 and Phase 1b trials, respectively, will begin in patients with both diseases next year. Ra has**a collaboration with Merck on an unspecified cardiovascular disease** and some other drug candidates in development, but none of them are in clinical testing as of yet.

Ra was formed in 2008, and has raised a total of $86 million in equity financing. (It's also gotten about $17.5 million so far through its partnership with Merck, and could get another $61.5 million in future payments.) Ra's most significant shareholders are New Enterprise Associates (22.09 million shares), Morgenthaler Venture Partners (17.38 million shares), Novartis Bioventures (17.23 million shares), Novo A/S (12.50 million shares), Lightstone Capital, and RA Capital Management (both 8.33 million shares).

Ra had $40.3 million in cash on hand as of June 30 and had burned through about $50 million since its inception. If it completes the IPO, Ra will trade on the Nasdaq under the symbol "RARX."

**Ben Fidler is Xconomy's Deputy Biotechnology Editor. You can e-mail him at bfidler@xconomy.com Follow @benthefidler**