

# Clarissa Desjardins - CEO, Clementia Pharmaceuticals, Canada

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10.11.2017

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*Clarissa Desjardins is the founder and CEO since 2011 of Canadian biotech success story, Clementia Pharmaceuticals. She explains the different milestones of her company and her priorities moving forward. She also gives her insights on the entrepreneurship environment in Canada and advice on how to make it even more compelling to increase the creation of biotech companies and become an international biotech hub.*

**Clementia is a Canadian biotech company focusing on two extremely rare diseases, fibrodysplasia ossificans progressiva (FOP) and multiple osteochondroma (MO). What made you decide to focus on these two areas?**

I had never heard of FOP prior to founding Clementia. It is an extremely rare disease characterized by the abnormal growth of bone in muscles, tendons and ligaments. People with FOP increasingly lose mobility and function, with most confined to a wheelchair by 30 years of age and with an estimated median lifespan of 40 years.

It was really a chance opportunity. I was working at a think-tank called the *Center of Excellence for Personalized Medicine* and collaborating with Roche on public-private partnerships. A senior executive at Roche pointed out a *Nature* medicine paper to me, which showed that a new class of molecules, retinoic acid receptor gamma (RAR- $\gamma$ ) agonists, were potent inhibitors of new bone

formation in animal models of this devastating disease, FOP. Roche had been working on a specific RAR- $\gamma$  molecule, palovarotene, for over ten years in a different area but they decided to abandon it.

What was needed was someone to start a company to finance clinical trials for this disease. As I already had experience in founding and managing biotech companies, I was approached by a Roche executive, who agreed to help facilitate the out-licensing of this molecule if I created a biotech company for it. Thus, I quit my job and started Clementia.

**Clementia had a significant milestone earlier this year with your August IPO. How will this advance Clementia's goal of developing effective new treatments for unmet medical needs?**

Clementia's IPO successfully raised USD 138 million in an upsized deal due to significant investor interest. These funds will enable us to complete two late-stage clinical trials: our Phase III trial for FOP, the last step of our clinical development program initiated four years ago; and a Phase II/III trial for a new indication, MO.

We are also hoping to file a new IND next year in a completely different field, ocular disorders. It turns out that our molecules are also anti-fibrotic in the eye.

We also have a very important data readout next year from our ongoing Phase II trial open label extension, where for the first time we treated subjects with FOP chronically instead of just during the time of the flare-ups. That data will be disclosed early-Q2 2018.

These are some of our near-term milestones.

**How significant is the unmet medical need in these areas?**

In FOP, there has never been a drug available, so I would describe the medical need as extreme. It is an exceedingly rare disease; 1.3 people per million have FOP. We are aware of approximately 15 patients in Canada.

For MO, it is much more prevalent – about one in every 50,000 – but this is also a disease of extreme unmet medical need with no treatment available. MO is a bone disease, caused by the same endochondral bone formations as in FOP, except that in MO, small bone tumors are formed around children's growth plates, causing a lot of pain and eventually loss of mobility. The only

treatment is surgery, so many patients go under one surgery a year, resulting in some patients undergoing around 20 surgeries in their lifetimes. It is another terrible disease.

**You mentioned a quite small size market with approximately 15 patients in Canada.**

**How do you expect that to affect Clementia's future development?**

Initially, the goal was to make a drug that had been abandoned by a pharmaceutical company and fund the clinical trials to see whether that drug would be efficacious. We do have Phase II data showing that palovarotene can reduce mean bone volume after flare-ups by approximately 75 percent. While that data was not statistically significant, it was sufficient for us to design our Phase III clinical trial and we are very optimistic about the potential for that clinical trial to have a positive clinical readout. This idea that "there is only a small patient population" was really secondary to that main priority.

The second consideration was if we were going to commercialize palovarotene ourselves and if we could make a return on investment in order to continue as a viable business. What is certain is that if we are successful, the market for FOP combined with MO is very interesting to investors and it ensures that Clementia will be a viable and self-sustainable biotech company in Canada for the long-term.

**Since 2011, the environment has evolved. What are your current biggest challenges and opportunities as well within the ecosystem here?**

Working within the rare disease space, you necessarily operate as a global company from the beginning. We have clinical trials and sites all over the world, including Japan.

Working in Canada has its benefits, for example, the talented workforce that we have here. We did open an office in Boston to recruit more senior executives as we could not find certain specific expertise here, for instance, like a Chief Medical Officer that had already developed a drug in the rare disease space. But for other positions, it was relatively easy to find what we needed locally. We currently have twenty people in Montréal and ten in Boston.

The other big advantage is the corporate tax rates, which are significantly lower in Canada than in the US. We did have some early issues with some Ethics Review Boards in certain hospitals, but we have been able to overcome these. Our biggest challenge was to negotiate the global protocol for

our Phase III clinical trial, as it had to be approved in the US, Europe and Japan, so that our patients would be treated the same way in each jurisdiction. That was what we had worked hard to accomplish over the summer prior to completing our IPO.

**Andrew Casey from BIOTECanada, the Canadian biotech association, highlighted that access to capital remains an issue in Canada. What policy measures could be taken to improve this for Biotech companies?**

Clementia did not have any trouble raising funds; we started with BDC Capital for an early-stage seed financing, and subsequently, most of our investors came from the US. We see an increasing openness from US-based investors to invest in foreign companies. More and more, they are realizing that there are great opportunities in Canada – and Montreal is only an hour's flight from New York or Boston, after all, so they can still be involved as much as they want in the governance of Montreal-based companies.

We do have quite a few local sources of funding now for biotech companies, but I know some companies still struggle. Large institutions in Canada are still not investing significantly in biotech. In terms of policies, it would be interesting to see how in Canada we could encourage the approach of allocating a small portion of large pension or mutual funds to biotech.

**Both industry and government have said they would like to see the other do more to invest in and promote R&D and innovation in Canada. From your perspective, have both sides done their fair share - and what more can be done?**

Most biotech companies already invest the vast majority of their funding in R&D so it is difficult for us to do more.

In terms of government financing, I do not believe the government should be involved in picking winners and losers. I am much more in favour of policies like R&D tax credits because I believe they are fairer, with the same rules applying to everyone. The best way for a government to promote innovation is to remove some of the barriers that exist and create policies that affect everyone equally.

From my perspective, a number of elements have to be in place for a healthy biotech ecosystem to evolve. The first is entrepreneurship. This has a lot to do with culture, individual motivation and

visible examples of success, which we need more of in Canada.

I also believe in incentives like lower income taxation because biotech is an inherently risky business. There is so much personal sacrifice involved in starting and managing a biotech company, so external incentives or disincentives really matter.

What is improving is the Canadian culture and the desire of young scientists to get involved in entrepreneurship, which is great. Certainly, the overall level of experience in managing biotech companies has grown tremendously. When I started my first company in 1992 as a graduate student, I felt like we were all learning the business from scratch. Today, there are numerous biotech veterans with experience either from creating or working in or selling biotech companies, which is hugely beneficial to the ecosystem at large.

**On a more cultural level, Canadians are often said to be too humble. How would you evaluate the Canadian entrepreneurial spirit?**

I believe that in this case, it is a numbers game – and there is simply an insufficient number of biotech companies of a critical size in Canada to showcase our true entrepreneurial spirit and spawn the creation of global biotech companies here. I remember one advisor telling me that we used to finance companies just enough to fail, and not enough to succeed. Statistically, there is a minimum level of financing required to move from one milestone to the next; there is no real shortcut. Therefore, a critical mass of funding going into a critical mass of biotech companies has to occur for global biotech companies to emerge here, but these conditions are now becoming real.

This idea that Canadians are maybe less ambitious or less bold may be true and I believe it goes back to the personal choices that we all have to make. The mentality seems to be, ‘do I want to conquer the world? Maybe not’. In the US, the sense is that people want to take their company as far as they possibly can, while in Canada, we tend to look for an exit – and often, an early exit. Is it because of the CEOs? Is it because of the investors? Is it because there are insufficient avenues for the company to grow?

In this business, it is ‘eat or be eaten’. I suspect that if we had more options for late-stage financing, we would be able to grow biotech companies that want to eat instead of be eaten and we are seeing this positive trend in other high-tech sectors.

While we punch above our weight in terms of our impact factor for basic research, science and publications, we have not explored this research to its fullest potential. When Canadian inventions

are known, it is usually because they have been commercialized by US companies. Scientific breakthroughs are not what drives economic activity. They do not create jobs or wealth unless these are translated into commercial activity, usually through biotechnology companies.

However, the environment is changing for the better and I fail to see why we cannot see great biotech hubs in Montréal or elsewhere in Canada.

**Coming back to Clementia, what profile of investors are you looking to attract?**

Both in Canada and in the US, we are looking to work with sophisticated healthcare investors that take a long-term view on the prospect for the success of our company.

**Do you have any advice for aspiring young entrepreneurs?**

My advice would be that it is possible to fulfil your goals; you are so fortunate to be born in Canada where everything is possible. I am a testament to that: without any independent wealth, starting off as a young woman with only my education and a business plan, I was able to raise millions of dollars for the fulfilment of my dream and the dreams of patients in need. This is something to be grateful for and to take advantage of, so if you have the capacity and a great idea, really go for it!

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