Bradley Campbell – President & COO, Amicus Therapeutics

Ultimately, we envision our competition as the disease rather than other companies

23.10.2020

Tags: USA, Amicus, Rare Diseases, Strategy, Access

Brad Campbell, president and COO of US rare disease specialist Amicus, talks domestic and global product launches, the company's first steps into the gene therapy space, and how a biotech success story like Amicus can be a guiding light in market access and pricing discussions.

Brad, could you share some highlights from your pharmaceutical career and of course your journey with Amicus thus far?

I started my career as a strategy consultant, before joining Bristol-Myers Squibb (BMS) in a sales and marketing role. After BMS, I went to business school and then officially began my rare disease career at Genzyme. That is really where I learned, from a career perspective, just how motivating and rewarding it is to be part of a patient-focused company working in cutting-edge rare disease therapeutic development.

I had known John Crowley, Amicus' founder, chairman, & CEO since my consulting days. He is a pioneer in the rare disease space and his incredible story began with the diagnosis of his two children with Pompe disease, a rare neuromuscular disorder, and his successive quest for a life-saving treatment. In 2006, John invited me to join him in building the next great orphan disease-focused biotech. The idea was that we would be technology agnostic and just focus on developing great medicines for patients and deliver great value for shareholders. When I joined, we were about 50 people, we were still a private company, and we were in an office park in rural New Jersey,

surrounded by mostly more office parks and farmland. Our lead investigational therapy was in Phase 1 development.

Today, we treat patients around the globe with a commercialized product, have a biologic in late-stage development, and are at the forefront of the future of medicine with a deep rare disease gene therapy pipeline. We have approximately 500 employees in nearly 30 countries across five continents, and we expect to reach USD 250-260 million in global revenues this year. It has been an exponential evolution from those early days but Amicus continues to be an entrepreneurial place where I work alongside innovative colleagues who are handpicked for their devotion to developing medicines that improve the lives of patients.

Looking at the US market, which in some ways can be as complex as all other markets taken together, what have been some of your insights regarding global strategies for successful rare disease product launches?

Our first commercialized product, GALAFOLD®, has a unique mechanism of action and provided us with a blueprint for how to conduct a successful drug launch globally. At Amicus, it is our mission to develop first-in-class or best-in-class therapies. GALAFOLD® is the first oral treatment for Fabry patients with certain amenable gene variants approved in the world. It was also the first small molecule in a space where the standard of care was an enzyme replacement therapy (ERT). This is a great advantage because of the ease of distribution, administration, and access – a benefit to both patients and the healthcare system.

We believe our initial success with this product is rooted in its ability to deliver meaningful value to patients and physicians alike. Testament to that, for example, is the fact that we have had an over 90 percent patient adherence rate since launch. We also aimed to price GALAFOLD® at a modest discount or parity to the current standard of care. This is not a market share or a classic discounting strategy; we want to ensure healthcare stakeholders think about the value of the product, not its price. For instance, we went through the UK National Institute for Health and Care Excellence (NICE) Highly Specialized Technologies (HST) evaluation process much faster than the industry average. The final evaluation document stated that our product had potentially better health benefits at a more affordable price. That was exactly the sort of value proposition we wanted to present, and this was what allowed us to enter various markets very quickly, reflected in our launch trajectory to date. We think that reflects the positive experience of patients and physicians.

Driving that success is a great team with a genuine and demonstrated passion for helping patients. The team brings world-class value in terms of how to compassionately support the patient community and how to deliver our therapies to those who need them most.

Critical to our success is a focus on market access. Our belief statement states that our medicines must be fairly priced and broadly accessible. Rather than focusing on the last dollar from a pricing perspective, we aimed to price our product at parity or at a discount to the current standard of care so that the physician, the payer, and most importantly the patient can focus on the value the product provides above all else. That kind of dedication to rapid and equitable access has proven to be incredibly successful by all measures.

Ultimately, we envision our competition as the disease rather than other companies. This creates a core of patient-focused, scientific dialogue that champions partnerships with physicians, payers, and patients. That trust and collaboration has been pivotal to our success and positions us as an industry champion of performance with high integrity. We demonstrate this collaborative approach by involving patients throughout the entire drug development pathway, bringing patient advisory boards in very early to provide input that helps determine things like the endpoints of a study and which patient-reported outcomes are most meaningful. When we speak to the US FDA, we always bring patient representatives. All these actions galvanize the dialogue around what is right and important for patients. Overall, this patient-driven strategy is something regulators have increasingly embraced in the last decade, incorporating the patient perspective throughout the regulatory pathway.

Many innovative US biotech companies find it daunting to establish commercial operations overseas despite having really novel products. But Amicus actually launched your product in Germany before the US and you have since established your international headquarters in the UK. What led to Amicus deciding to chance it on international expansion?

Necessity is the mother of invention. It just took longer for us to cross the finish line in the US, and we knew we wanted to evolve into an international commercial company, so we put major efforts into pursuing Europe. As our regulatory pathway unfolded, GALAFOLD® was first approved in Europe and we launched the product in Germany in 2016 with subsequent approvals in other geographies and approval in the US in 2018.

What led us to establish an international presence, and what served as a model for success in rare disease drug launch, all came down to the belief we had in our people and in the talent that we could grow. At Amicus, we have always worked diligently to garner top tier talent whose excellence is inspired by our unique story in rare disease – the passionate entrepreneur phenotype – we knew that the Amicus team could reach as many countries as quickly as possible because of, not only a great product, but the right workforce to move it forward.

We believe that the best people to bring our treatments to patients are Amicus employees. We hired our first commercial employee in the same year leading up to our launch of GALAFOLD®, investing energy in expanding Team Amicus rather than mobilizing a disjointed international infrastructure for hire. I went over to London to help build our international organization, and it was certainly an adventure. We focused on attracting passionate entrepreneurs with extensive industry experience, who would approach their job with the same mindset as if he or she had a loved one or child who lives with a rare disease, but who was still mindful of our company's resources. That balance of passion and entrepreneurial mindset drove our early and continued international success, even though we are often a third or even a quarter of the size of our competitors in any given market. We are now in over 30 countries in the world, the vast majority through our own direct Amicus salesforce.

How has COVID-19 affected your communications and dialogues with HCPs and other stakeholders?

The industry is certainly looking at this very carefully as we are all navigating uncharted territory. For Amicus, I am pleased that we have managed to bring the same level of scientific rigour to our virtual dialogues with HCPs and collaborators as we did in person so that we continue to add value to the conversation. It has, however, raised the bar for what productive conversations mean so we are working hard to ensure that we bring something new and interesting that can support our joint efforts to fight rare diseases.

For instance, we recently held our scientific meeting, Fabry Connections, virtually for the first time. Over 250 physicians from all around the world joined, which is even greater than the in-person attendance last year. As always, the agenda has been driven by the physicians and the initial feedback has been incredibly positive.

Even in a remote context, the good news for us is that we have been working in the Fabry and Pompe space for around a decade or so and our network is quite well-established. We are lucky to

be in a digital age with extensive virtual tools, hopefully, they will continue to improve and enable advancement across the industry to maintain existing relationships and build new ones.

Amicus has also decided to enter the gene therapy space. What was the rationale behind entering this space and having shared some of the pros and cons of building connections in the times of COVID-19, how will you establish your stakeholder network in this new area?

Gene therapy is a logical next step for us. GALAFOLD® is a pharmacological chaperone that focuses on targeting protein trafficking within the cell with natural enzyme production. We developed a keen understanding about the importance of these mechanisms, glycobiology and protein trafficking. When we thought about tackling Pompe disease, we realized that that chaperone was not sufficient on its own and we applied our knowledge around protein engineering, trafficking and targeting to externally produced enzymes. That led to AT-GAA, our investigational ERT combined with an enzyme stabilizer.

With that strong expertise in metabolic disorders, we started thinking about where we wanted to focus our energies next, and we decided to look at the areas of greatest unmet need. It was a natural evolution for us to apply our protein trafficking and engineering approach to gene therapy. We entered a partnership with the University of Pennsylvania, which provided Amicus with disease-specific worldwide rights to Penn’s next-generation technologies. The team at Penn is one of the leaders in the field and they bring incredible capabilities in gene therapy and gene delivery. Their gene therapy capabilities paired with our protein engineering expertise helps ensure that the enzyme being expressed by the gene therapy is able to get where it needs to go. We believe very few in the industry are looking at this approach, and this kind of strategic partnership will allow us to take on the challenges of drug development even during the pandemic.

Symbolic of our collaborative mission, we unveiled our Global Research and Gene Therapy Center of Excellence in Philadelphia in March of 2020. This 75,000 square foot state-of-the art research facility across from Penn not only strengthens our collaboration, but it also significantly expands Amicus’ R&D capabilities to advance our rare disease gene therapy programs as part of a thriving innovation hub. Amicus chose Philadelphia for its well-regarded ecosystem for biotechnology and gene therapy research that offers an ideal environment for Amicus to advance its pipeline, attract and retain top talent, and foster external collaborations in rare disease.

In terms of developing relationships in this new space, I think it also comes down to the quality and innovation of our products and our focus on driving the science. Most of the diseases we are focused on have no approved therapeutic options. For instance, we have clinical programs for multiple forms of devastating rare conditions like Batten disease for which no treatment exists. Children with Batten disease start to lose their ability to walk, speak and think during childhood, eventually pass away in late childhood or early adulthood. If we have novel products and are guided by great science, we can attract physician-scientists and patient organizations to work together as collaborators. Our strategy has had to expand to accommodate our new virtual world but its core is the same – continue to collaborate with the best innovators and utilize the best tools.

Other recurring thematics in the US are market access and pricing. What is the impact that a biotech company like Amicus can have on this topic?

I think we can take a leadership position and be a shining light to guide our industry in bringing value to patients.

A significant place to start is with pricing and access. Amicus has made a pricing promise with five core components. In addition to pricing our therapies at parity or a modest discount to existing standards of care, we have also pledged to never raise our prices above the Consumer Price Index (CPI), which is particularly important for payers to build their pharmacoeconomic impact models. We have also pledged to invest a portion of our revenue back into research until there’s a cure for the diseases we are addressing. In addition, we commit to providing lifetime access to medicines for any clinical trial patient. We currently have a number of Fabry disease patients that continue to receive our medicine, in many cases for over a decade, because we have not achieved reimbursement for GALAFOLD® in their markets yet. Finally, we pledge to provide patient services and financial support for patients that cannot access our medicines. We have expanded our Bridges program, where we provide our drugs for free to patients in countries where we know we are not going to pursue reimbursement.

You have worked at Genzyme during your career and it is seen as a rare disease biotech success story. As Amicus continues to grow at a fast pace, how will you retain the personality and character of the company?

Like our industry peers, our people and our patient-focused culture are our most valuable assets. That culture is woven throughout our business strategy and our day-to-day operations. Our singular mission has united us during the global pandemic and helped us continuously adapt to a changing world. We have to hold on to that. The more we grow, the higher the risk of diluting that culture, but so far, one of my proudest achievements at Amicus is being able to retain our focus on developing medicines for people living with rare diseases and keeping the patient perspective ever present.

I hope to see Amicus continue to evolve into the next great biotech company with many patients served by our therapies. If in five years from now, there are 5000 patients around the world living with a rare disease who are treated with an Amicus medicine, we would be truly successful.

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