Stealth BIOTHERAPEUTICS | Leading Mitochondrial Medicine

October 1, 2021

Members of the Barth syndrome community:

August 2021 saw the achievement of a shared milestone – the first submission of a new drug application for elamipretide by our team at Stealth, representing the first new drug application for any investigational product for Barth syndrome. This was a Herculean effort by our small team – a new drug application is a massive undertaking even for large companies – and one of which we are exceptionally proud. While we await the FDA's decision whether to review our application (and there is no guarantee that the FDA will review it), I am reminded of the path that led us to this juncture. We would not have reached this point in the FDA process without your voice, your inspiration, and your tireless advocacy and support for the work needed to develop therapeutics for this critical medical need.

BSF leadership first approached us in 2014 to ask that we consider initiating development efforts in Barth syndrome. Quite frankly, we had reservations about the request; it is inordinately difficult to develop drugs for ultra-rare conditions not least because there are so few patients available to participate in clinical trials. Your leadership team persisted, proposing preclinical work with physician scientists dedicated to Barth syndrome research, which we undertook. I recall an early event in Boston at the Paul S. Russell Museum of Medical History and Innovation where Kate McCurdy spoke of the trajectory of the disease and the unmet medical need; attending my first BSF International Scientific, Medical and Family conference in 2014; and watching the first Patient-Focused Drug Development Meeting for Barth syndrome in 2016 (from a hotel room, while traveling on business, with a twelve-hour time difference!). Your leadership team – Kate, Emily, Shelley, your Board, and your scientific advisers – helped further our scientific interest and introduced us to the challenges of the disease. Your community's passion, drive, and optimism, and your love of all your Barth brothers (and sisters!), all underscore the reason we are in drug development in the first place − to help patients in search of potential therapies .

Whatever the outcome of our shared journey from a regulatory perspective, I want you to know that your courage in participating in clinical research and, as importantly, raising your voice and telling your story has resonated beyond your small community. When I speak to my colleagues at our company meetings, I tell your stories. When I speak to scientific and medical thought leaders about our programs, I describe Barth syndrome. You are small, but you are mighty. Your families and members of the BSF leadership team have accompanied us to meetings with the FDA, you have bravely told your stories on social media, and you have made yourselves heard in an unimaginably courageous and compelling way. You've been inspiring and amazing to work with and have invigorated our commitment-to-purpose on this long and sometimes rocky journey to discover and develop therapies for ultra-rare diseases.

You are truly exemplary of strength and solidarity through adversity. We are deeply grateful for your support and partnership.

Warm regards,

Reenie McCarthy
Chief Executive Officer