Lemur Biotech Part 1:

Richard Fossi enjoyed kayaking. In recent months, the tributaries around the Outer Banks of North Carolina seemed to be the only place where he could escape the constant interruptions he experienced when in his office at Lemur Biotech. On Fridays in the summer, Richard often left the office early enough to reach the beach in time for a quick paddle before sunset. That was going to be important today. Richard had a lot on his mind, and he hoped he could use the time on the water to finalize a plan.

A Curious Malady Experienced by Lemurs—and Humans

Potentially fatal, Stress Induced Respiratory Crisis, StRIC, is a genetic condition that is estimated to affect somewhere between 12,000 and 15,000 individuals in the United States. Prior to a serendipitous discovery at the Duke Lemur Center, patients suffering from StRIC were often misdiagnosed as severe asthmatics. In 2008, Dr. Artemis Buena-Todos had conducted genetic research in the quiesco lemurs. Dr. Buena-Todos was searching for a genetic cause for a curious behavior of the quiescos. When exposed to a sudden loud noise—including claps of thunder in North Carolina's frequent spring storms—the animals would experience a sudden tightening in the left superior bronchus. In many circumstances, the animals would immediately pass out. Researchers had observed that this behavior would have quickly led to extinction of the quiesco had it not been for their near lack of predators on their native Babaomby Island.

Dr. Buena-Todos identified a pathway that linked the sudden burst of adrenaline to bronchus constriction. A story about the "lemur pathway" in Duke's student newspaper, The Chronicle, had caught the eye of Dr. Glenda Groundswell at the medical center. She was the one that made the intellectual leap of using Dr. Buena-Todos' research to better understand patients who suffered an abnormal level of respiratory distress when placed in a stressful environment. With Buena-Todo's pathway in hand, Dr. Groundswell was quickly able to identify the genetic link and isolate the StRIC patients.

If allowed to progress to a crisis event, StRIC patients experience excruciating chest pain along with the psychological trauma of surviving an experience that closely resembles drowning. The only course of treatment is to be placed on mechanical ventilation devices. Hospital length-of-stay periods can extend to four or five days.

Inhibiting the Prime8 Protein

Richard Fossi had been acting as an executive in residence at Tar Heel Life Ventures (THLV), a moderately sized venture firm operating in Research Triangle Park. Placing Richard as the CEO, THLV had formed Lemur Biotech to build on Dr. Groundswell's discoveries. Within two years, the company had developed an inhibitor for Prime8 a key protein involved in the Buena-Todos pathway. Formulated as an IV infusion, the product, LEM-001 was expected to be administered to StRIC patients experiencing a crisis who presented at hospital emergency rooms.

Taking advantage of the 2012-2015 surge in biotechnology investment, Lemur had independently funded a progression of successful proof of concept trials. Richard had assumed that the anticipated follow on funding would be relatively easy—there had certainly been plenty of interest in 2014 when the company completed its IPO.

His recent meetings with potential investors had not been the "champagne and roses" that Richard had expected. While he knew investors would quiz him on clinical issues, Richard had been caught off guard by the relentless questions on patient access, price, epidemiology, and the frequency that those patients experience crisis. Investors wanted to know if payers would require genetic confirmation of StRIC before they would allow treatment with LEM-001. They also wanted to know what price Richard anticipated charging for the product. Moreover, several potential investors probed Richard's expectations regarding the entry of competitive products—a young member of one due diligence team even asked Richard whether he thought it was wise to invest in a product to treat a monogenic condition when it was just a matter of time until gene editing technologies eliminated the patients' problems altogether.

The Realities of Pricing and Competition

On reflection, Richard had to admit that it was short-sighted to simply expect investors to congratulate him on the remarkable clinical success of LEM-001 and open their checkbooks. The issues they were raising had, of course, occurred to him—even worried him on certain sleepless nights—but he simply had not had time to prepare structured analyses.

At first, pricing had seemed the least of Lemur Biotech's worries. StRIC was unquestionably an orphan condition, and the benefit of LEM-001 was expected to be profound. The Phase 2 study had shown reopening of the bronchus in less than six hours. Even with the anticipated observation period, the length of stay was expected to fall below two days. Some trial patients had even expressed that they should have been permitted to go home soon after administration.

Despite these benefits, Richard had been told that the only reasonable initial pricing for LEM-001 would be equivalent to the cost savings to the hospital under the DRG (diagnosis related group). Applied broadly across the United States, the DRG provided hospitals with a set fee for a patient's "episode of care." From this perspective, the value of LEM-001 was only the reduction in the length of stay in the hospital for a StRIC patient who experienced a crisis. Faced with the prospect of an unreasonable ceiling being placed on the price of LEM-001, Richard was exploring whether it would be possible to gain a "carve-out" payment from payers. In a carve-out, a separate fee would be paid to the hospital to reimburse for use of LEM-001. While Richard had received some positive feedback regarding the possibility of a carve-out, everyone who seemed to know about this issue warned him that pursuing a carve-out would be a long process and success was certainly not guaranteed.

There were two other companies pursuing treatments for StRIC. While clinical leaders in those companies did not have the easy access to Dr. Buena-Todos that was enjoyed by Lemur, they seemed to be making strong early progress. One of these companies, RareifiedAir, was working on an inhibitor of Prime8 that would be delivered using a nebulizer. Although the phase 2 program was still being designed, Richard had heard rumors that RarefiedAir's product was being designed to allow patients to carry the nebulizer with them, and self-administer a rescue dose. Even if successful, their program would launch three to four years after LEM-001.

Another company, LiberBronch, was in even earlier stage development. Richard had heard that LiberBronch had received angel funding for a low dose oral Prime8 inhibitor that would be taken as a prophylactic therapy. However, so far, the company had been operating in stealth mode and Richard could not be sure of the veracity of any of these reports.

Traffic had been surprisingly light and Richard reached the Outer Banks even earlier than he had planned. Immediately upon arrival he changed his clothes and walked purposefully to the dock. Within minutes he was on the water. His thoughts, however, remained concentrated on how to address the many questions that had been raised in his investor meetings.

This case study is fictional and developed solely for the purpose of stimulating thoughtful discussion at the Biotech CEO Summit. Any similarity to any company or individual is merely coincidental.