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COMPANY UPDATES

(At left, Christine McSherry--founder of the Jett Foundation, named for her son who has Duchenne Muscular Dystrophy--consoles the "troops" Monday night outside Washington, D.C. after the latest FDA panel smack down of Sarepta's Eteplirsen.)

A fairly new Member asked me in an e-mail Tuesday morning, essentially, what the big deal is with my (and several other newsletter writers') "devotion" to **Sarepta Therapeutics.** This especially, he wondered, as Eteplirsen would have a very limited population of patients to treat. I can't speak for other writers, but will answer from my own perspective.

Though over the 20 years now (officially, as of the coming July issue!!) that I have offered individual stock recommendations among the other content of *The National Investor*, health care stocks generally have not been among the best-represented sectors. There's no one particular reason for that. However, I first became animated with a company called AVI Biopharma (renamed Sarepta Therapeutics several years ago) back in the late 1990's for several reasons.

Not the least among them is my belief--and *hope* to a great extent--that science is not only on the verge of more effective treatments for some diseases, *but cures*. This has long been a very personal interest of mine. My oldest son turned 35 years old last month; yet way back in 1997 was facing such a dire prognosis from a rare metabolic disorder that he--and our entire family, 10 of us!--ended up accompanying him for a trip arranged by the Make A Wish Foundation. That he continues to live (though he is unable to live/function on his own) is a continued blessing to our family and all that know him.

Three other of my eight children have Type One diabetes, even though neither their mother nor I do, and there is no existence of that in either of our families. This over the years has increased our interest in finding causes of this. . .finding alternative treatments, diets, etc. to better cope and more. I often look into different therapies and such that are coming down the line where diabetes is concerned.

Suffice it to say that I "root" for anything I think is legitimate that may one day lead to less of the kind of challenges that have been faced by my family...and by millions of others'. For present purposes, though--where this newsletter is concerned--I need to find "stories" that have at least some kind of justification investment-wise as well.

The "old" AVI (and present-day Sarepta) was among the first biotech companies I learned of to be developing therapies/drugs based on RNA-targeting. If you've not done so before I encourage you to visit the company's web site to learn more of this (at http://www.sarepta.com/technology/rna-medicine) as they can explain this WAY better than I can. Suffice it to say that I saw this technology as the next major step forward in treating and perhaps one day reversing the causes of some diseases. On that basis, I first recommended AVI Biopharma as a speculative "story" stock nearly 20 years ago. As you longer-tenured Members remember (or should!) we have been in and out of them on several occasions.

Our most spectacular foray into AVI, though--which changed its name to Sarepta during this time--culminated in the moon shot these shares experienced in the latter part of 2012. That was when *one of* the company's RNA-targeting drugs, Eteplirsen, showed its first major early clinical progress in being a treatment for DMD, Duchenne Muscular Dystrophy. *Keep in mind that, when I added the company again to my recommendations prior to this (when its shares were in the $1 - $2 range once more) I did NOT have my eye on this specifically.* Instead, I saw that this company had once again fluttered back down to an attractive entry point (as volatile biotech issues often do) and I thought it wise again to have access to the wide array of potential breakthrough drugs the company is working on (which, as I reminded you in my most recent re-recommendation, can be learned of at http://www.sarepta.com/our-pipeline)

Abetted by the monstrous financial returns that then-owners of SRPT shares enjoyed, *the added human interest dimension* catapulted Sarepta--and Eteplirsen--into the consciousness of an entire investment community that had previously never heard of the company. Not one of you reading this is unfamiliar with the decades-long Jerry Lewis Muscular Dystrophy Association telethons and the long, emotional quest to one day find a cure for "Jerry's kids." **As I wrote back after Eteplirsen's first big splash in 2012, this was (and STILL IS) the closest thing to that yet.**

As I watched the 11-hour Food and Drug Administration Advisory Committee hearing this past Monday, I was incredulous (with many others, most important the DMD patients, families, care givers and others who were part of the record-breaking crowd for such a hearing) that what seemed to matter to the 13-member panel relatively little was **the evidence IN THE FLESH in front of them that Eteplirsen works!** The attitude of a majority of the AdComm panel members was of such an overly formulaic and technical nature that some seemed incapable of recognizing the human beings in front of them that were

living testimony to Eteplirsen's efficacy. Somewhat fittingly, one of the panelists who ended up voting "no" on the final question on the drug's effectiveness *actually gave* as his reason for voting against the drug the rationale that, "The drug definitely works, but the question was phrased differently." Such was the often-tortured and *inhuman* process as carried out by some of the AdComm members.

Though it may well be the longest of long shots, the F.D.A. can still reject the AdComm's recommendation and grant the NDA for Eteplirsen. This would not be the end of the challenges; the company would be continuing broader trials (one of which, a Phase 3 trial, is currently underway but won't be completed until at least next year, if not 2018.) But it would make Eteplirsen available to many more DMD patients NOW *who will most likely die without it.*

Some are putting their hopes in the seemingly encouraging stance of the F.D.A.'s Dr. Janet Woodcock, who more than once during the hearing seemed to reveal a softer stance personally on Eteplirsen. Other than the commissioner, she will reportedly be the most senior person voting on the drug by or before May 26 when the agency's decision on Eteplirsen's application is rendered. Among other things, she spoke of the error of *not* approving a drug that has shown at least some effectiveness (and is SAFE, a matter not in any dispute with Eteplirsen unlike its two competitor drugs, both of which have already been rejected by F.D.A.) when such disapproval will mean DEATH for possible patients.

After all, Congress' will is for accelerated approval of any drug where there is little to no downside risk--and potential life-saving upside--*when that drug will meet now-unmet needs*. That, we believe, is deserved by Sarepta's Eteplirsen. Well over 100 Members of Congress and Senators have made these wishes known specifically regarding Eteplirsen to the F.D.A. *The first of 52 speakers in the public testimony part of Monday's hearing, in fact, was Congressman Mike Fitzpatrick (R-PA.)* Rather than this laudable **and Congressionally-mandated** goal what we got instead from a small majority of the AdComm members was a disposition, as one blogger put it, ". . .not to seek relief for patients, not to err on the side of doctors in the field, but to err on the side of hurdles. "

Losing another battle, DMD patients, families, doctors and advocates all will naturally continue the fight. There's no other option. And I have no doubt that the F.D.A. will be relentlessly pressed to discard the AdComm's recommendation; perhaps with some threats from favorable Members of Congress who will see a rejection of Eteplirsen as a conscious rejection of a congressional mandate by the F.D.A.

From an investment standpoint, the wild ride will continue. This company's public shares have *already* been among the Top-10 of the most profitable ones I have recommended over 20 years. My belief is that more profits will come down the road, even if a rejection of Eteplirsen's NDA on or before May 26 means we have to wait longer.

SRPT will assuredly remain volatile. As one *CNBC* personality, professional trader Jon Najarian, quipped yesterday (Tuesday), the company from an investment perspective is "the gift that keeps on giving." After predictably gapping down at the open (SRPT was halted all day Monday for the hearing) to the $7.00/share area it fought back all day from that initial low to close well above $11.00/share. Will it see $7 again, if the May 26 decision confirms the AdComm recommendation and the NDA for Eteplirsen is turned down? *$70* if that finding is rejected and the F.D.A. grants the application? I don't know.

I continue to recommend Sarepta not just for the more mercenary reasons exhibited (perhaps unintentionally) by Najarian. Those reasons--if we are looking simply at my job of finding interesting, potentially profitable opportunities for you, my Members--would be sufficient.

My "devotion" to this company and the broader story goes beyond that, though. Maybe it's because having two sons with different diseases who for all I know may not be here tomorrow helps me to identify with those DMD patients and families in this instance. Maybe it's because I, as so many others, would enjoy seeing the valiant Jerry Lewis witness the first big breakthrough drug to successfully treat *any* form of muscular dystrophy get approval--and benefit more patients than those few in trials--before *he* dies.

To me, when we can combine investment returns (or at least potential, carefully-vetted) ones with a good cause that benefits PEOPLE and saves/increases their life, I can't think of better types of opportunities to know about. . .root for. . .and participate in, if appropriate.

That goes not just for Sarepta and the promise of slowing down or arresting the devastating progression of DMD. It goes for a disease--Alzheimer's--which is in the crosshairs of **Anavex Life Sciences (NASD-AVXL)** and its work. I can already say that, through this company, the foundational cause of *many* central nervous system and metabolic disorders, including my oldest son's, are now even better understood by me. And though Members have already logged some quadruple-digit gains with AVXL also, it's a story--and cause--worth sticking with.

Finally, having had a mother who died at too young an age after a quarter-century battle with cancer, I am naturally desirous of seeing any company that could better the chances of people in the future to beat that dreaded disease. That's why I am animated by my most recent recommendation in the biotech space, **Theralase Technologies (TSXV-TLT; OTC-TLTFF.)** Their novel approach to selectively destroy cancer cells in patients (in their first clinical trials, they are targeting patients with Non-Muscle Invasive Bladder Cancer) is more fully explained in my recent Special Report on the company, at http://nationalinvestor.com/wp-content/uploads/Theralase-Technologies-Special-Report.pdf

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