REGENXBIO Inc.

Ticker: RGNX

I know what you are thinking. You are about to read another biotech report with more acronyms than words you know the definitions. I promise you this report is written in layman's terms and I'm certain you will find it informative and understandable. You do not need a PhD or MD to understand anything in this article however, before you dive in, I urge you to watch this 2-minute video as it will help lay the foundation for what follows : http://www.regenxbio.com/pages/navtechnology/index.htm.

Business Description

REGENXBIO Inc is a biotechnology company. It is engaged in the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy (Morningstar). RGNX has a market cap of $300 million and is based out of Rockville, MD. If you visit their webpage they pride themselves on being the leader in AV (adeno-associated virus) Gene Therapy. They have also been licensed by Biogen to support its Regenex vectors specifically for retinal degenerative diseases caused by single gene defects (inherited sight degenerative diseases that have no known cure). RGNX's current revenue stream is being used to fund research and development of its internal product pipeline. They do not expect it to be reoccurring or a significant portion in the future as they will be able to market and sell their internal drugs for more.

Thesis

RGNX is a small cap biotech company leading the industry in AAV gene therapy. Their five internal drugs are key to becoming successful. They have more than twenty other drugs, technologies, and processes that are being licensed out to produce a steady stream of capital to fund their internal drug research. RGNX is able to fund its research from recurring payments from its licenses. Two thirds of RGNX's market cap is held in cash, their cash burn is less than average at $60 - $70 million for 2016, and they have little to no long term debt and lease obligations. RGNX's management stated in their 2Q earnings call that their cash should last them through 2018. I like the fact that the furthest any of their drugs

have made it to clinical trials. This may make their stock a speculative play but it also means that their stock is trading at much lower prices. Any results, positive or negative, whether in R&D, preclinical trials, or clinical trials will cause large and immediate reactions with respect to their stock price. RGNX's balance sheet, cash flow, and income statement look stable and have a positive outlook assuming nothing material changes. In addition, management had a very optimist outlook on the future of their NAV technology and AAV Gene Therapies on their first ever conference call that took place on August 9th.

About AAV Gene Therapy

In layman's terms, AV Gene Therapy is the use of a very small non-disease virus to affect both dividing and non-dividing cells (as you saw in the short video clip).

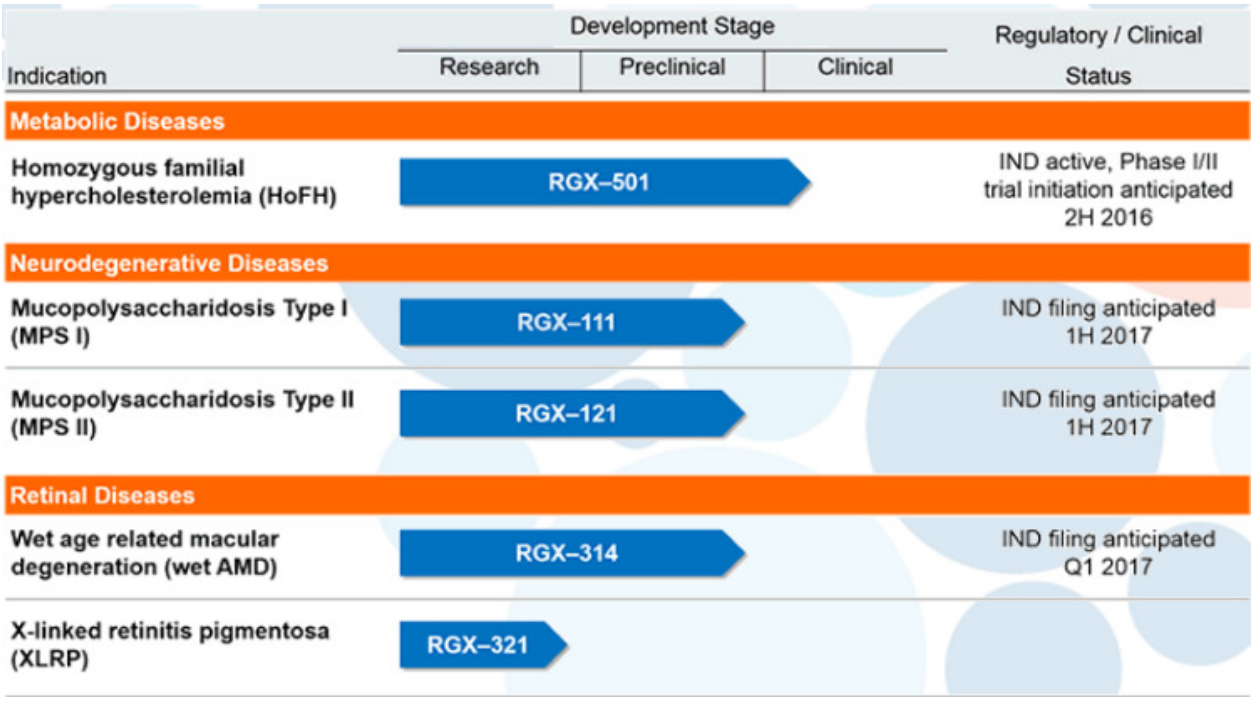
Why AAV Gene Therapy?

AAV Gene Therapy can insert genetic material at a specific site on chromosome 19 with near 100% accuracy. Chromosomes are the inherited instructions that make us who we are. All of our chromosomes can be found at the nucleus in each cell. If there is an issue on the chromosomal level it has been inherited and will not correct over the length of your life. Chromosome 19 represents almost 2% of our total DNA and likely contains about 1,500 genes that provides instructions for making proteins (U.S. National Library of Medicine).

Why is AAV Gene Therapy Important?

- Targeting is nearly 100% accurate
- Can affect both dividing and non-dividing cells
- Known to cause little to no immune response (body will not reject it) and will not cause a disease

REGENXBIO Programs



IND filing stands for Investigational New Drug. This filing is reviewed by the FDA and decides whether or not the product is safe for the company to start clinical trials where it will be tested on humans. The clinical trials must be completed in order to bring a drug to market. This can cost upwards of hundreds of millions of dollars and potentially take years.

RGX – 501 (Clinical/ Orphan Drug Status)

Purpose – Treat LDLR (low density lipoprotein receptor) gene that causes high cholesterol

Target Market – Inherited HoFH disorder that effects 1 and 160,000 (very rare)

Source: The FH Foundation

RX – 111 for MPS I (Clinical/ Orphan Drug Status)

Purpose – Treat MPS Type I (Mucopolysaccharidosis Type I). It is a genetic disorder that resides in the central nervous system

Target Market – Inherited trait that affects around 1,000 patients born each year worldwide (very rare)

Source: National MPS Society, RGNX 2015 10-K

RX – 121 for MPS II

Purpose – Treats MPS II, also known as Hunter syndrome which affects the central nervous system

Target Market – Inherited trait that affects 1 in 100,000 to 1 in 150,000 males

Source: National MPS Society

RGX – 314

Purpose – Treats wet age-related macular degeneration (wet AMD) which affects blood vessels in the eye and causes severe vision loss

Target Market – 11 million people in the United States have this condition, but it only affects 10-15 percent of them.

Source: AMD.org, American Society of Retina Specialists

RGX – 321

Purpose – Treats retinitis pigmentosa (RP) which is the most common inherited form of blindness

Target Market – 100,000 patients in the U.S. alone

Source: REGENXBIO, Blindness.org

It is very important to note that none of RGNX's drugs have been brought to market yet. AAV Gene Therapy has not been proven to work although in some instances according to biotech companies there have been "promising observations" (NCBI). RGNX could be the first to prove efficacy for this type of therapy. Now you may ask how is it that a $300 million small cap biotech company from Maryland is leading this research with support from Biogen? The answer is pretty simple and straight forward. AAV Gene Therapy has not been studied extensively because AAV is unlike other viruses used for gene

therapies in the past. AAV does not because a disease in the human body and since it was different, researchers never gave it the time of day. It was only until the 2000's when researchers discovered the useful properties of AAV Gene Therapy. RGNX was founded in 2008 and has specialized in AAV Gene Therapy ever since. They are on the forefront of this innovation and already have over a hundred proprietary methods already in the hands of researchers.

Benefits of Treating Rare Conditions

- Orphan Drug Tax Credit (ODTC) – Tax credit on 50% of qualified clinical trial costs for orphan drugs. This tax credit starts as soon as the drug is deemed an "Orphan Drug" by the FDA and ends when the FDA approves it for patients.
- Orphan drugs are often approved quicker
- Seven years of protection compared to the five years that conventional obtain
- Ability to control the pricing because patients have few if any other options

INTERNALLY DEVELOPED PRODUCT CANDIDATES

Indication	Development Stage: Research / Preclinical / Clinical	Regulatory / Clinical Status	Commercial Rights
Metabolic Diseases			
Homozygous Familial Hypercholesterolemia (HoFH)	RGX-501	IND active, Phase I/II trial initiation anticipated 1H 2016	REGENXBIO
Neurodegenerative Diseases			
Mucopolysaccharidosis Type I (MPS I)	RGX-111	Phase I/II trial initiation anticipated mid-2016	REGENXBIO
Mucopolysaccharidosis Type II (MPS II)	RGX-121	IND filing anticipated 1H 2017	REGENXBIO
Retinal Diseases			
Wet Age-related Macular Degeneration (wet AMD)	RGX-314	IND filing anticipated 2H 2016	REGENXBIO
X-linked Retinitis Pigmentosa (XLRP)	RGX-321		REGENXBIO

NAV TECHNOLOGY LICENSEE PRODUCT CANDIDATES

Indication	Development Stage: Research / Preclinical / Clinical	Regulatory / Clinical Status	Commercial Rights
Central Nervous System			
Spinal Muscular Atrophy Type I	AVXS-101	Phase I	AveXis
Mucopolysaccharidosis Type IIIA (MPS IIIA)	LYS-SAF302	Phase I/II	Lysogene
Amyotrophic Lateral Sclerosis (ALS)	VY-SOD101		Voyager
Friedreich's Ataxia - CNS	VY-FXN01		Voyager
Huntington's Disease	VY-HTT01		Voyager
Mucopolysaccharidosis Type IIIA (MPS IIIA)			Esteve
Friedreich's Ataxia - CNS			Annapurna
Hematologic / Liver Diseases			
Hemophilia B	DTX101	Phase I/II	Dimension
AIAT Deficiency	ANN-001	IND active	Annapurna
Hemophilia A	BAX 888		Baxalta
Hemophilia A	DTX201		Dimension/Bayer
Crigler-Najjar Syndrome Type I	AT342		Audentes
Glycogen Storage Disease Type Ia (GSDIa)	DTX401		Dimension
Ornithine Transcarbamylase (OTC) Deficiency	DTX301		Dimension
Undisclosed			Dimension
Undisclosed			Dimension
Undisclosed			Dimension
Severe Allergy	ANN-004		Annapurna
Muscle Diseases			
Pompe Disease	AT982		Audentes
X-linked Myotubular Myopathy	AT132		Audentes
Friedreich's Ataxia - Systemic	ANN-003		Annapurna
Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)	AT307		Audentes
Friedreich's Ataxia - Systemic			Voyager

RGNX's licenses its NAV technology drugs (bottom 2/3 of diagram) to other companies and in return different fees and payments including milestone payments, royalties, and upfront fees. They then use this capital to further their internal drugs (top 1/3 of diagram) research which will be worth hundreds of times as much if successful.

Recent Events / Potential Catalysts

RGX-501

- Began clinical trials in the U.S. in November of 2015

RGX-314

- Plan to begin production in 3Q 2016 for clinical trials that they expect to start in the first quarter of 2017 after IND approval

RGX-121

- Plans to file for IND approval in the first half of 2017
- Plans to begin production for clinical in fourth quarter 2016

RGX-111

- Plans to file for IND approval in the first half of 2017

License with Biogen

- REGENXBIO has granted Biogen a worldwide research license for two drugs that treat rare genetic vision disorders
- Biogen recently entered into an agreement with UPenn for their gene therapy and gene editing technologies. The agreement entails a $20 million upfront payment, payment for R&D over the next 5 years, milestone payments of up to $137.5 million per product as well as royalties. If Biogen's agreement with RGNX is anything like this, there could be a huge opportunity for shareholders

Source: Value Line, RGNX 10-K, Seeking Alpha, RGNX Website

Risks

- Time and cost of development
- Future success hinges on 5 product candidates, 2 of which have only made it to clinical trials
- Disconnect between success of preclinical trials and clinical trials
- FDA, EMA regulatory risk
- Clinical approval

- Obtaining orphan drug approval before competition
- Significant competition

Source: RGNX 10-K

Analyst Price Targets

- Zach's Investment Research Median Analyst Estimates - $33
- Morgan Stanley - $36
- Chardan Capital - $35
- Piper Jaffray - $36

Closing Summary

I rate RGNX as a buy. I believe we should average in up to $5,000 on 5 separate occasions because of the volatility in the stock price. The future looks very bright for this company. RGNX only needs 1 of their 5 internal drugs to be successful in order to more than double their market cap. Rare diseases have become an extremely profitable business over the past 5 years due to new rules and regulations by the FDA and RGNX is on the forefront with its innovative AAV gene therapy technology. Their revenue stream from licensing deals should keep them afloat until one of their drugs makes it to market. I suggest we trim several biotech positions to free up capital in our healthcare sector allocation to invest in RGNX.

Eric Bayer
Portfolio Manager, Towson University Investment Group
Ebayer1@students.towson.edu

Sources

REGENXBIO's Website – www.regenxbio.com

Journal of Gene Medicine - http://www.abedia.com/wiley/vectors.php

U.S. National Library of Medicine: Genetics Home Reference - https://ghr.nlm.nih.gov/chromosome/19

NCBI - http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2570152/