



COMPANY / TICKER:	ABEONA THERAPEUTICS (ABEO)
MARKET CAP:	\$535 MILLION
CURRENT SHARE PRICE:	\$11.45
REVENUE	\$0.2 million per quarter
CASH / SHARE	\$3.05 per share (incl. Oct 2017 capital raise)
EXPECTED SHARE PRICE:	\$4-6 (AT LEAST 50-60% DOWNSIDE)

Summary

- In Oct 2017, shares of ABEO hit a new high of \$19.55 following the release of seemingly positive data in its clinical trial for MPS-III.
- ABEO quickly used that strength to raise money in an equity offering at \$16 in October.
- But when additional data was released last week, ABEO quickly began to plunge every day.
- “Smart money” investors now realized that Cohort 1 data had been badly manipulated to show optimal results.
- Out of a three person Cohort, one patient was given an arbitrary “floor score” for a cognitive test – precluding any real chance of further declines in cognitive ability. Another patient was removed from the trial altogether.
- This information was not made clear in October at the time of the equity offering
- In addition, Cohort 2 data was visibly mixed, with the strongest results coming from a disputed test method and with very negative indications coming from the industry standard test
- The leading industry journal specifically recommends two different tests than the one being used by ABEO. The non recommended test is the one which analysts continue to cite as indication of strong results.
- The investors who have figured this out have been selling heavily every day since that data was released on Feb 8th.
- Yet sell side analysts continue to put positive spin on the results by focusing on a single “outlier” to explain negative results.

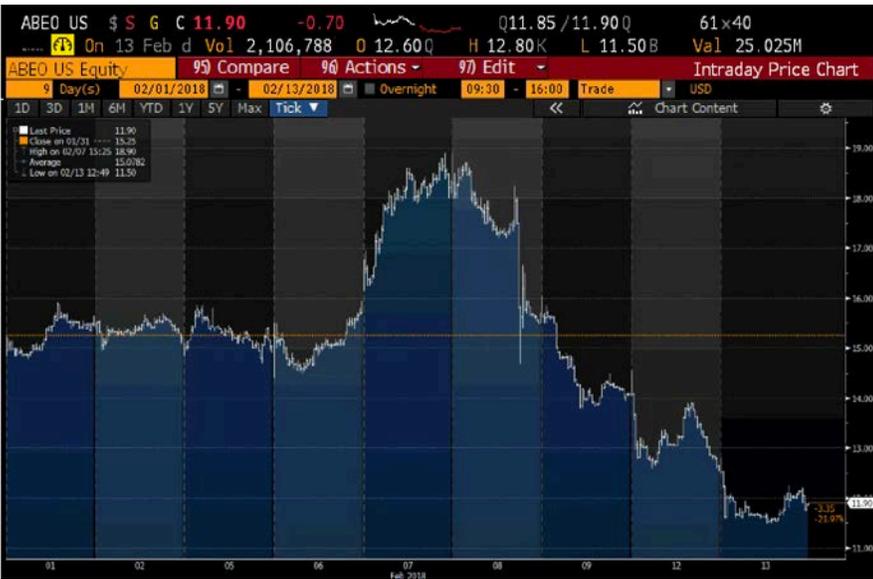
Abeona: How SOME of Wall Street Missed the Manipulated Data

This report is the opinion of the author. It is not a recommendation for anyone anywhere to do anything at any time. Do your own research, form your own opinions. The author is not an investment advisor. The author is short ABEO. The author may conduct transactions on various securities mentioned in this report (or on securities of competitors of other comparable companies, securities etc.) within the next 72 hours.

ABEO Share Price Plunges Despite Strong Analyst Support

On February 7th, Abeona’s share price reached a 2018 YTD high of \$18.90. But following the release of its MPS-III data on February 8th, stock began to immediately plunge. Sell side analysts immediately came out in support of Abeona, saying that the data released did not justify the sell off. It should be noted that all of the bullish analysts had just acted as underwriters on Abeona’s recent equity offering and collectively shared in \$5 million in fees.

The key argument raised by these analysts was that any perceived weakness in the results was only due to a single outlier within a small sample set. Overall data, they say, remains positive. There may even be a possibility that data will show an uptrend as Cohort 2 matures.



But as we can see from the chart below, despite the very vocal enthusiasm from sell side analysts, Abeona’s share price has traded straight down on heavy volume every day since that data came out. The share price has now fallen by 30% even as these analysts continue telling us it should double.

Joint Book-Running Managers

Jefferies	RBC Capital Markets
	<i>Lead Manager</i>
Cantor Fitzgerald & Co.	
	<i>Co-Managers</i>
FBR	H.C. Wainwright & Co.
JonesTrading	Maxim Group LLC

Prospectus supplement dated October 16, 2017

February 8, 2018

Abeona Therapeutics Inc.

ABO-102 WORLD MPSIIIA update: selloff a buying opportunity

Selloff on ABO-102 MPS-IIIa update is a buying opportunity with Cohort 2 neurocog variability explained away by illness in single outlier.

“Smart money” investors have now discovered that data from ABEO’s Cohort 1 has been manipulated to deliver the best possible result for one of the three patients. Then a third patient (out of three) was removed entirely. This “data” is therefore entirely nonsensical. This manipulation was not made clear to investors ahead of the equity offering in October. Once investors discovered this, the stock began plunging.

A pattern among plunging biotech stocks

Below you can see a table of past biotech stocks that I wrote about at MoxReports.com.

At their peaks, each of these stocks had investors salivating about their further prospects for triple (and even quadruple) digit gains.

A consistent theme among biotech stocks is that complicated information can be manipulated.

To read my full article on each of the biotech stocks below, click on the name in the far left column.

Name	Ticker	Date	At time of article		Current		Change
			Price	Mcap (\$m)	Share price	Mcap (\$m)	%
OHR Pharmaceutical	OHRP	1-Jul-14	\$9.08	\$225.8	\$0.31	\$17.4	-96.6%
Tokai Pharmaceuticals	NVUS	2-Nov-15	\$10.98	\$248.0	\$0.39	\$24.9	-96.4%
Northwest Biotherapeutics	NWBO	7-Jul-14	\$6.71	\$399.3	\$0.33	\$104.2	-95.1%
Galena Biopharma	GALE/SLS	12-Mar-14	\$3.25	\$383.1	\$0.18	\$8.3	-94.4%
CytRx Corporation	CYTR	12-Mar-14	\$4.78	\$265.6	\$0.29	\$47.5	-94.0%
Regulus Therapeutics	RGLS	19-Nov-14	\$16.26	\$790.3	\$1.13	\$117.5	-93.1%
Inovio Pharmaceuticals	INO	27-Mar-14	\$14.56	\$872.2	\$4.06	\$366.6	-72.1%
Advaxis	ADXS	21-Jan-15	\$8.37	\$227.7	\$2.70	\$111.5	-67.7%
Keryx Biopharmaceuticals	KERX	11-May-15	\$10.70	\$1,108.5	\$4.28	\$510.3	-60.0%
ZIOPHARM Oncology	ZIOP	21-Oct-12	\$5.00	\$398.1	\$3.88	\$550.8	-22.4%
Revanche Therapeutics	RVNC	20-Nov-15	\$35.31	\$990.9	\$29.15	\$901.7	-17.4%
Nymox Pharmaceutical	NYMX	10-Aug-16	\$3.55	\$161.3	\$3.25	\$170.2	-8.5%

In nearly every case above, bullish sell side analysts were calling for these stocks to double or triple, just before they plunged to a fraction of their original price.

As with ABEO, I highlighted significant problems with each of the biotech stocks above. Yet many investors insisted on riding these down to near zero on the belief that “the market for such a drug would be huge”, “the affliction itself is horrible”, or simply that “much of the data looks good”.

Abeona: Company Overview

Abeona Therapeutics (ABEO) is a clinical stage biotech company focused on gene therapy for rare disorders. It was founded around 2012 by current CEO [Tim Miller](#) and former Chairman [Al Hawkins](#). In May of 2015 PlasmaTech [acquired](#) Abeona for about [\\$34mil](#) in stock and performance milestones. This transaction was effectively a reverse merger that brought Abeona public. The company is based in Dallas, TX.

The primary focus of Abeona is ABO-102 and ABO-101, both of which are being evaluated for treatment of Mucopolysaccharidosis (MPS) type III (also known as Sanfilippo syndrome). Nationwide Children's Hospital (NCH) employees Haiyan Fu, PhD and Doug McCarty, PhD [developed](#) ABO-102 and ABO-101 and Abeona licensed the IP from NCH. NCH is conducting clinical trials for ABO-102 and ABO-101 and they also conducted the Natural History Study (NHS) being used as the control cohort in the trials. Both ABO-102 and ABO-101 are currently in Phase 1/2 clinical trials for MPS IIIA and MPS IIIB.

Abeona is also developing EB-101 for recessive dystrophic epidermolysis bullosa ("RDEB") (also known as "butterfly syndrome") which causes large open wounds and blisters. EB-101 has completed Phase 2 trials and is currently in hopes of going in to Phase 3 going forward. While EB-101 is somewhat further along the regulatory pathway than Abeona's other drugs (ABO-102 & 101), analysts have only recently considered its potential and most have not modeled for it. This is likely due to the small addressable market, questionable efficacy, and competitive market for skin grafts.



Additional early stage pre-clinical programs include EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for infantile Batten disease (INCL), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using CRISP/Cas9-based gene editing for rare blood diseases.

Following the presentation of MPS-III data in October of 2017, shares of Abeona reached \$19.95 (more than quadruple their 2017 lows). Shortly thereafter, on October 19th, Abeona issued \$92 million of stock in a new equity sale at \$16.00.

During 2017 shares of ABEO rose by as much as 4x as a result of "good data". However, Abeona did not reveal the manipulation of patient data in collection of other scores. When problems began to emerge in Feb 2018, ABEO share price immediately tumbled. ABEO is still up by 200% vs. 2017 lows.

Recent developments – 2018

January 29th – Abeona received Regenerative Medicine Advanced Therapy [designation](#) for EB-101. This is an expedited program similar to Breakthrough Therapy designation, which this drug already has.

February 7th - Abeona reported initial 30-day [safety and biopotency data](#) for the first patient enrolled in Phase I/2 for ABO-101 in MPS IIIB. The estimated enrollment in the program is nine patients in two cohorts.

February 7th – Matthew Herper of Forbes released an interview with gene therapy pioneer [Jim Wilson](#). Wilson has recently become concerned with toxicity in monkeys and piglets treated with high doses of adeno-associated virus (AAV). AAV is the same method that Abeona utilizes to deliver ABO-102 & ABO-101 into patient tissues. Although some public concern has resulted from his finding ultimate consequences are currently unclear.

February 8th – Abeona reported [top-line data](#) from Phase 1/2 trial in MPS IIIA at the WORLDSymposium. The data was presented by lead investigator Kevin Flanigan, MD of Nationwide Children’s Hospital. Following the release of this data, ABEO’s share price quickly fell by 30% over the subsequent several days, despite positive commentary from analysts.

February 12th – Abeona received [Orphan](#) drug designation for ABO-202 for infantile Batten disease, a fatal lysosomal storage disease of the nervous system. ABO-202 is currently preclinical and human trials have not yet begun.

Evaluation criteria for treating MPS-III

MPS III (Sanfilippo syndrome) is a genetic disease which causes enzyme deficiencies that result in the abnormal accumulation of glycosaminoglycans (sugars) in body tissues. The incidence of MPS III (four types A-D combined) is estimated to be 1 in 70,000 births.

In MPS III, the predominant symptoms occur due to accumulation within the central nervous system (CNS), including the brain and spinal cord, resulting in cognitive decline, motor dysfunction, and eventual death. To date, there is no cure for MPS III and treatments are largely supportive.

In attempting to evaluate effectiveness against MPS-III, Abeona's clinical trial is measuring two categories of responses.

Biological responses measure things like the hoped-for reduction of Heparin Sulfate or GAG levels which remains in the cerebrospinal fluid ("CSF") or in the urine. These are the sugars whose accumulation results in the damage shown above. In addition, the trial will look for increased enzyme levels as well as reduced liver and spleen volumes.

Phase I/II gene transfer clinical trial of scAAV9.U1a.hSGSH for Mucopolysaccharidosis (MPS) IIIA

ClinicalTrials.gov: NCT02716246

- Phase 1/2 open-label, dose-escalation clinical trial
 - Cohort 1: 5 X 10¹² vg/kg (n=3 subjects): ages 6.5, 7.0 and 5.4
 - Cohort 2: 1 X 10¹³ vg/kg (n=3 subjects): ages 2.9, 2.5 and 3.2
 - Cohort 3: 3 x 10¹³ vg/kg (4 patients enrolled): ages 5.2, 2.3, 4.9 and 8.0
 - will enroll 4-5 additional patients at three global sites: US, Spain and Australia

Primary Outcome	Determination of safety based on the development of unacceptable toxicity; defined as the occurrence of two or more unanticipated Grade III or higher treatment-related toxicity.
Secondary Outcomes	<ul style="list-style-type: none"> • Reduction in CSF and/or urinary HS and/or GAG • Increase in CSF and serum SGSH enzyme activity levels • Reduced liver and spleen volumes at 6 and/or 12 months after treatment, as measured by magnetic resonance imaging (MRI) • Improved adaptive functioning, or arrest of decline in adaptive functioning, as assessed by parent report using the Vineland Adaptive Behavior Scale • Improved cognitive ability or arrest of cognitive deterioration at 6 and/or 12 months after treatment, as measured by direct testing of the child using the Leiter International Performance Scale and the Mullen Scales of Early Learning

The biological responses can be seen as a useful indicator. In addition, they are able to be measured precisely. **However the far more important metrics are the external/observable tests that indicate a cessation or slowing decline of mental and behavioral impairment.**

To assess changes in adaptive functioning (behavior), Abeona uses the Vineland Adaptive Behavior Scale. To assess cognitive ability Abeona uses the Leiter Scale.

Cohort 1: Leiter Scale data so deeply manipulated as to be entirely useless. The supposedly "positive" data then touted as indicating success. Cohort 2: Leiter data again has been positioned as very positive, even though it entirely contradicts the results of Vineland Scale. [Orphanet Journal of Rare Diseases](#) recommended against Leiter. MPS-III competitors (Lysogene and Shire) use "more robust" tests by industry.

The Leiter Scale: Why It Was Chosen. Why It's a Problem

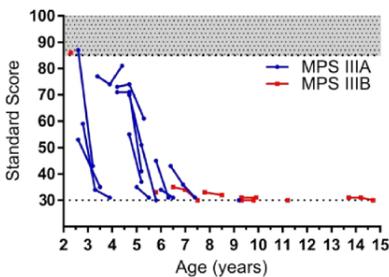
To evaluate results against a control arm, Abeona is comparing behavioral and neurocognitive progression against a Natural History Study (“NHS”) which tracked the deterioration of MPS-III patients. That study was conducted by Nationwide Children’s Hospital (NCH). Kevin M. Flanigan, MD of NCH is listed as an [author](#) of a paper that reviewed results of the NHS. He is also the lead investigator that recently [presented](#) the updated results at the WORLDSymposium on February 8th. This implies that the lead investigator of ABO-102 had significant control over patient selection for the Natural History Study and the clinical trial that it is compared against. He also happens to have [financial ties](#) to companies including Sarepta Therapeutics (SRPT) and PTC Therapeutics (PTCT).

It's also worth noting that ABO-102 and ABO-101 are based on IP that was [created](#) by Haiyan Fu PhD and Douglas McCarty PhD. Both are employees of Nationwide Children’s Hospital and Dr. McCarty is on Abeona’s [Scientific Advisory Board](#). They are also listed along with Dr. Flanigan as members of the NCH team that was responsible for recruiting patients for Abeona’s Natural History Study ([slide 4](#)).

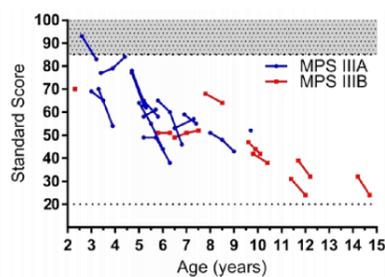
The [charts](#) below show the deterioration suffered by MPS-III patients in the NHS. On the left is the Leiter Scale, which measures cognitive ability (nonverbal IQ). **NOTE** the range for “normal” (i.e. unaffected) children is shown at the very top in shaded grey. That range runs from around 87 on up. You can then see the deterioration in cognitive ability for each of MPS IIIA and MPS IIIB sufferers. There are two points to notice. First, the rate of deterioration (slope) is very rapid. Second, the “floor score” for the Leiter-3 scale is a score of 30. The subjects in the study do not go lower than this.

Clinical Trial Readiness: The NCH MPSIII Natural History Study

- **25 subjects: 15 MPS IIIA and 10 MPS IIIB**
- Assessments at 0, 6, and 12 months
- Established standards for biochemical (serum and CSF) studies and volumetric (MRI) relevant to the clinical trial
- Validated neurocognitive and behavior outcome measures for MPS III clinical trials



Leiter-3 Nonverbal-IQ Score over 6 months.
Normal range of unaffected children is shown shaded.



Vineland Scale - Behavior Assessments over 1 year
Normal range of unaffected children is shown shaded.

Truxal et al (2016) Mol Genet Metab, 119(3):239-248

5

If a patient is assigned a “floor score” of 30, it is not possible for them to show any further deterioration in their scores. A steady state amounts to success.

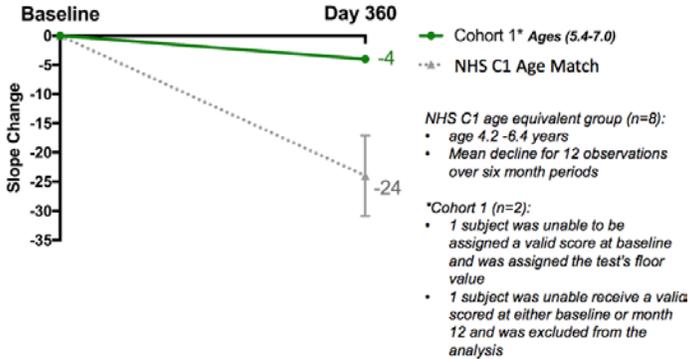
Also the data points on the Vineland scale on the right (measuring behavioral assessments) tend to be more spread out and declining at a slower rate.

Below I show how in Cohort 1, the Leiter data in Abeona’s trial has been completely manipulated guaranteeing a better result, regardless of any real world deterioration. And in Cohort 2, it is again the Leiter data that is being widely touted as evidence of a strong clinical result for MPS-III patients (even though this data is directly contradicted by the Vineland data).

Here is why all of this matters. [The Orphanet Journal of Rare Diseases \(OJRD\)](#) specifically recommends two different tests for testing neurocognitive assessment in Sanfillippo syndrome (MPS III) patients. The recommended tests are Bayley Scales of Infant and Toddler Development (3rd edition) or the Kaufman Assessment Battery for Children (2nd edition). **The Journal specifically does not recommend Leiter.** Competitors Lysogene and [Shire](#) Pharmaceuticals have conducted clinical trials that utilized these other tests (i.e. not Leiter) for neurocognitive assessment.

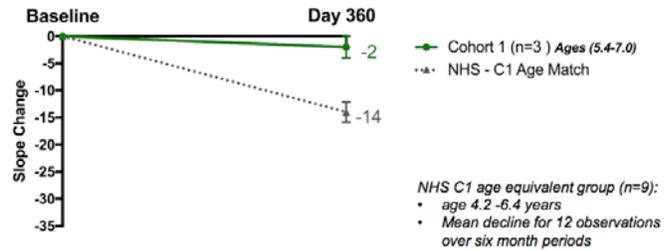
Cohort 1 – Manipulated data now meaningless

Cohort 1: Evidence for Neurocognitive Stabilization (Leiter) Compared to Natural History Study at 1 Year Follow-Up



16

Cohort 1: Stabilization (Vineland) of Adaptive Behavior Compared to Natural History Study at 1 Year Follow-Up



18

As soon as the latest data was presented, we saw the beginning of a steep and steady decline in the share price. Despite the chatter from the sell side analysts, I suspect that this plunge had nothing to do with any “outlier”. Instead, it becomes apparent that in Cohort 1, the data which was being portrayed as “strong” was actually utterly meaningless. We can now see that one of the patients in Cohort 1 was assigned a “floor value” because he could not be accurately measured. And then one other patient was removed entirely. So out of a 3 person Cohort, only one was left with a legitimate score. By assigning a “floor value” to that patient, it meant that there was little if any possibility of recording any decline in cognitive ability – in other words, the gene therapy would appear to be yielding a tremendous result.

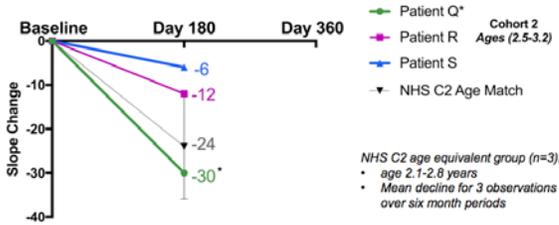
As far as any presentations I can see, this arbitrary “floor score” was not made clear to investors ahead of the October equity offering, But it is clearly quite material. We can now see the immediate reaction from investors, who have driven the stock down by 30%. Investors who bought in to that October equity offering at \$16 are now collectively underwater by around \$25 million.

There are other problematic details worth noticing. The Leiter data from Cohort 1 only contains a single point (-4), even though it is described as covering two observations. In other presentations Abeona has either shown each data point separately or provided a range like they did for NHS data in the very same chart. (Notice the “High/Low” bar around the number -24 on the same chart). Had they broken out the two data points separately (like they do on every other chart) the implications of this “floor score” would have been immediate obvious to even a casual observer. The data is truly meaningless, but it gets worse.

Here’s why the stock is selling off: A few savvy investors now realize that cohort 1 data is effectively meaningless. Had this data been previously disclosed, the stock never would have hit the highs of 2017 and 2018.

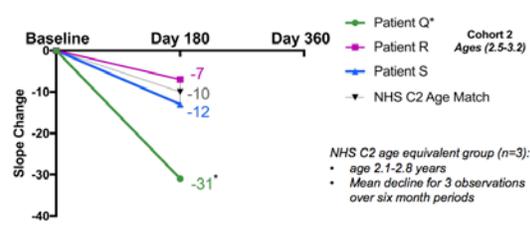
Cohort 2 – Leiter Score Deviates Again

Cohort 2: Neurocognitive Stabilization (Leiter) in 2 of 3 Subjects Compared to Natural History Study at 6-months Follow-Up



17

Cohort 2: Adaptive Behavior (Vineland) Compared to Natural History Study at 6-months Follow-Up



19

Just like Cohort 1, Cohort 2 focuses on two tests, a neurocognitive test (Leiter) and a behavioral test (Vineland). The results of the Leiter test could be viewed to be encouraging, with some modification. One of the three scores in Cohort 2 is described by sell side analysts as a visible outlier. With the remaining two Leiter scores outperforming the NHS control, analysts expressed the view that the data is strong.

But again, there is a reason why Leiter is not the industry recommended test for MPS-III. Once we look to the right at the Vineland Score, we can see a very different result. Yes, we can again see a noticeable outlier. But we can also see that this outlier is now so wide that even very aggressive corrective efforts would bring it nowhere near the control arm. Then we can also see that even a second patient also performed worse than the control arm. And the one single patient that performed better than the NHS control did so by a tiny margin of just 3 points (as compared to 18 points on the Leiter scale to the left.)

So in other words, the Vineland data on the right provide strong indication of little or no effectiveness being seen in Cohort 2.

In Cohort 1 we saw the outright manipulation of data by arbitrarily assigning the “floor score” which would then result in optimal apparent performance. But in Cohort 2, all we see is an over reliance on a test that is not recommended (but which shows positive results), while simultaneously ignoring the test that is industry standard (but shows basically zero effectiveness).

The non recommended test provides positive results, while the industry standard test provides dismal results. And then analysts tout the “encouraging results” from Cohort 2.

Who is currently selling ABEO ?

As sector enthusiasm for gene therapy heated up, a number of “smart money” biotech funds began taking stakes in Abeona. However, it is notable that these funds took their stakes at much lower share prices. They have also consistently limited their stakes to less than 5% of Abeona, so that they can retain the flexibility to immediately sell their shares at any time without having to disclose. Note that Knoll Capital sits at 5.04% so is just marginally over that limit.

(Note: SCO Capital owns 30% of Abeona. SCO is the investment vehicle of Abeona’s chairman Steven H. Rouhandeh, who was previously an investment banker and Wall St. Attorney before Abeona and its predecessor firm.)

ABEONA THERAPEUTICS INC								CUSIP 00289Y1											
1) Current		2) Historical		3) Matrix		4) Ownership Summary		5) Insider Transactions		6) Options		7) Debt							
Search Name		All Holders, Sorted by Size		21) Save Search		22) Delete Search		23) Refine Search											
Text Search				Holder Group		All Holders		Investment Manager View											
24) Color Legend				Shrs Out		46.8M		% Out		98.35		Float/Shrs Out		65.71		SI % Out		18.62	
Holder Name	Portfolio Name	Source	Opt	Position↓	% Out	Latest Chg	File Dt												
1. SCO CAPITAL PARTNERS LLC		Proxy		14,094,660	30.13	650,001	04/10/17												
2. VIKING GLOBAL INVESTOR...	VIKING GLOBAL INVE...	13F		4,040,727	8.64	1,802,007	12/31/17												
3. # FMR LLC		13G		3,903,591	8.35	0	12/29/17												
4. KNOLL CAPITAL MANAGEME...	KNOLL CAPITAL MAN...	13F		2,359,767	5.04	0	12/31/17												
5. # STATE STREET CORP		ULT-AGG		2,256,787	4.82	1,918,635	12/31/17												
6. # ADAGE CAPITAL MANAGEME...		ULT-AGG		2,082,300	4.45	82,400	12/31/17												
7. BAKER BROS ADVISORS LP	BAKER BROS ADVISO...	13F		1,984,364	4.24	300,000	12/31/17												
8. # BLACKROCK		ULT-AGG		1,868,378	3.99	245,217	12/31/17												
9. WELLINGTON MANAGEMENT...	WELLINGTON MANAGE...	13F		1,497,437	3.20	1,289,952	12/31/17												
10. # VANGUARD GROUP		ULT-AGG		1,310,058	2.80	293,193	12/31/17												
11. CASTLE HOOK PARTNERS LP	CASTLE HOOK PARTN...	13F		1,190,100	2.54	-179,506	12/31/17												
12. # T ROWE PRICE GROUP INC		ULT-AGG		734,238	1.57	525,270	12/31/17												
13. CORMORANT ASSET MANAG...	CORMORANT ASSET M...	13F		575,000	1.23	175,000	12/31/17												
14. AHN MARK J		Form 4		493,182	1.05	149,500	11/08/16												

Warren Buffett > Quotes > Quotable Quote



“If you've been playing poker for half an hour and you still don't know who the patsy is, you're the patsy.”

– Warren Buffett

tags: poker-patsy

Read more quotes from Warren Buffett

Cost basis for reported shareholders is 40-50% below current levels

			History Details
ADAGE CAPITAL PARTNERS GP LLC			USD
Est. Holding Period (yrs)	1.25	Cost Basis/Share (LIFO)	7.14
Current Mkt Val	23.84M	Cost Basis/Share (FIFO)	7.14
First Holding Date	12/31/16	Cost Basis/Share (Average)	7.14
KNOLL CAPITAL MANAGEMENT LP			USD
Est. Holding Period (yrs)	3.00	Cost Basis/Share (LIFO)	7.04
Current Mkt Val	27.02M	Cost Basis/Share (FIFO)	7.04
First Holding Date	03/31/15	Cost Basis/Share (Average)	7.04
BAKER BROS ADVISORS LP			USD
Est. Holding Period (yrs)	1.50	Cost Basis/Share (LIFO)	8.08
Current Mkt Val	22.72M	Cost Basis/Share (FIFO)	8.08
First Holding Date	09/30/16	Cost Basis/Share (Average)	8.08
CASTLE HOOK PARTNERS LP			USD
Est. Holding Period (yrs)	1.25	Cost Basis/Share (LIFO)	7.03
Current Mkt Val	13.63M	Cost Basis/Share (FIFO)	7.86
First Holding Date	12/31/16	Cost Basis/Share (Average)	7.70

What about that “outlier” ?

Clearly RBC was looking at the same data presentation that I was, but somehow their analyst came to the conclusion that the data was great if we could just ignore a single outlier. RBC made no mention of the fact that Cohort 1 data on Leiter was now totally meaningless. In that three person Cohort, one patient was removed, while the other person was given an arbitrary “floor score” from day one such that there was zero possibility of showing any cognitive decline, regardless of what happened in the real world. As a result, 50% of a tiny N=2 sample size was now legitimate, while the other 50% was artificially “perfect”. So again, if RBC even noticed this then they failed to mention it to us.



RBC Capital Markets

February 8, 2018

Abeona Therapeutics Inc.

ABO-102 WORLD MPSIIIA update: selloff a buying opportunity

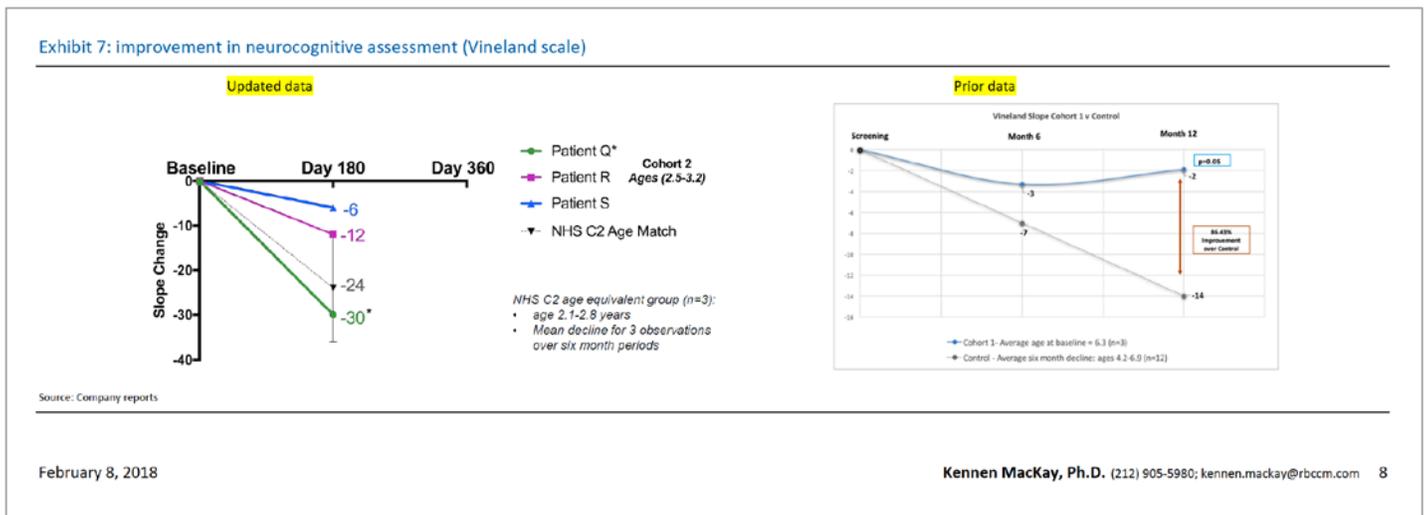
Selloff on ABO-102 MPS-IIIA update is a buying opportunity with Cohort 2 neurocog variability explained away by illness in single outlier.

Instead, RBC focused on a single “outlier” stating that because one patient was sick on the day of measurement, the data was unfairly skewed. We already saw this earlier in our analysis of Cohort 2. Yes, indeed, if we take out this outlier from our Leiter data in Cohort 2, then the data does look favorable. But then when we look at Vineland data, results become a disaster. Even performing a very aggressive correction for the “outlier” still shows basically zero effectiveness for Cohort 2 on this scale.

For their conclusion, RBC states:

“We see the existing data from neurocog scoring systems at multiple ABO-102 doses (cohort 1 and 2) suggesting potential for benefit, and look to longer term follow up to resolve concerns surrounding the single patient outlier driving increased variability. See Exhibits 6 & 7 for details.”

But in concluding ABEO’s promising future, **RBC is actually using the wrong data.** Below is a screenshot from RBC. You can see clearly that the data presented as “**Vineland Scale**” is actually the data that we saw earlier for the “Leiter Scale”. This is important. The Leiter scale does indeed look quite positive (if we first exclude an outlier) while the Vineland scale still looks disastrous even with any correction for any “outlier”. RBC has therefore assumed that the “disastrous” data is now the “positive” data. Please feel free to look back to the [MPS III presentation](#) from February 8th.



Conclusion – the Sell Side Says “BUY” while the Buy Side Screams “SELL”

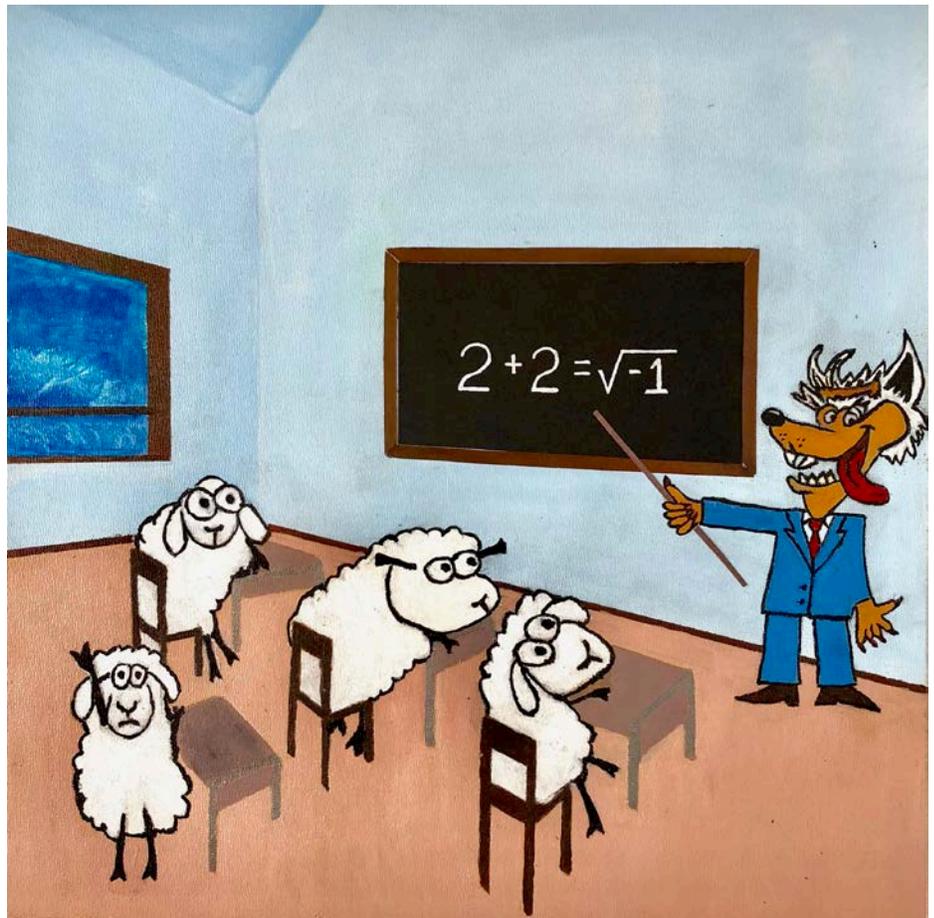
Immediately following the presentation of the most recent MPS-III data, sell side analysts rushed to express their optimism and support for Abeona. They assured the public that the data behind Abeona’s MPS-III trial was quite positive. Yet any close read of the data shows us that much of the Cohort 1 data is meaningless, while the most recent Cohort 2 data is downright bad.

In contrast to the optimism of the analyst / bankers, Abeona’s share price tells us a very different story. Immediately after the data came out, the share price began to plunge. And it has continued plunging with little support every day since.

It is very easy for sell side analysts to simply regurgitate some predetermined view on a stock, even as they ignore facts and data which present obvious problems.

The “smart money” investors read through the data and then form their own conclusion.

With Abeona’s share price down 30% since that data was released, it is already quite clear what the smart money is thinking (and doing).



If you don't know, it's OK to raise your hand !

Some dissenting opinions on those stocks above

Looking back to my past Biotech reports on page three, I had revealed deep problems which were so visible that they should largely not have been debatable. Yet many investors seek to cast blame anywhere they can and then insist on riding a failed investment down to zero.

8790041
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It doesn't sound like you listened to the conference call. Look at the lines of vision improvement. You also did not mention the case

roosterly9
Comments (53) | + Follow | Send Message | Mute
Mr. Pearson, you are yourself a type that will be void of conscious. You make a living by and depriving them of capital raising opportunities. Promotions are very very wrong, but to write

blueridgeco
Comments (14) | + Follow | Send Message | Mute
Gale is a victim of short sellers, and low life stock manipulators. I am long, believe in the company and product and have really never experienced this type of capitalist manipulation to the harm of a good company and product potential. Think they got to phase 3 trials on songs, dance bs? I doubt it. Think the management does not have a right to sell shares. Think marketing your company and product is a crime. That's a great concept. Perhaps now we can now see TV, print and internet commercial free.

libouban
Comments (169) | + Follow | Send Message | Mute
This author practices the arcane art of shorting. Just have a look at his previous work under the author's name and look at the articles written so far, they all ().

jessielucky
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The management of CYTR is shoddy but the data is real. That is the big difference between GALE and CYTR.
13 Mar 2014, 10:15 AM | Report Abuse | Like 9 | Reply

dnricoli
Comments (5) | + Follow | Send Message | Mute
Inovio was just recognized with Best Therapeutic Vaccine award at World Vaccine Congress. First in Class for Cervical Pre-Cancer Immunotherapy and Best Early Stage Biotech.
27 Mar 2014, 02:38 PM | Report Abuse | Like 9

mr.investor
Comments (191) | + Follow | Send Message | Mute
I am long INO because of its science and potential. Excellent short article though. Great read and I can tell you really did your research.
27 Mar 2014, 10:18 AM | Report Abuse | Like 14 | Reply

pennywatchdog
Comments (895) | + Follow | Send Message | Mute
ARNA has a drug that is completely optional for overweight people, completely optional, with many other options out there to choose from to help one lose weight.
KERX has a drug that is absolutely necessary for all CKD patients on dialysis, with no other options, period.
We have heard so much from you ikarus, and nothing from the supposed author of this imaginary problem, maybe you are one and the same?
01 Jun 2015, 10:44 AM | Report Abuse | Like 0 | Reply

libouban
This author practices the arcane art of shorting. Just have a look at his previous work under the author's name and look at the articles written so far, they all ().
back is part of what G. Hudson describes rather well in his
...CO...
Lots of FUD, nothing on the market potential, the potential of saving lives, of a cancer treatment, etc.

Ultimate failure. If the data were good, there would be no need to manipulate it in the first place. Yet many investors insist on holding their shares to zero. Common justifications include “the market potential is huge”, “it is such an awful disease”, “data looks great” and “I believe in company and product”.