



Therapeutics for rare
and severe diseases



January 2023


LEADERSHIP

			
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ADVISORS & PARTNERS

			
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BOARD


<p>Georgia Erbez, BA <i>Director</i></p> <p>COO Walking Fish Therapeutics Prior CFO, Raptor</p> <p>UC Davis BA</p>

Medical and business model similar to Raptor, but for larger population

BIOMARIN[®]

Mkt Cap \$15.5 billion

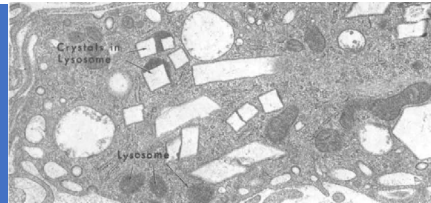
raptor[®]

Sold for \$800 million

AltiBio

CYSTINOSIS:

genetic disease resulting from a dysfunctional cystine transporter in the wall of lysosomes



Cystine crystals in lysosomes¹

- Systemic disease (“starting in kidney”)
- Multiple medical needs
- Thiol therapy gold standard
- US target patient population: 500

[REDACTED]:

genetic disease resulting from [REDACTED]

Image Redacted

- [REDACTED]
- Multiple medical needs
- [REDACTED] therapy gold standard
- US target patient population: 5,000

1. Published in Investigative ophthalmology 1970
[Intralysosomal cystine crystals in cystinosis](#). Vernon G. Wong, T. Kuwabara, R. Brubaker, W. Olson, J. Schulman, J. Seegmiller



SEVERE PATIENT DISSATISFACTION WITH [REDACTED]

“

"It makes me nauseous and I have to take with food and even then I still get nauseous from it." 10/2021

"I have tried twice on the old formula and once on the new one and the side effects were worse each time. I also had problems with nausea which seems to be a big one..." -10/2021

"Been on [REDACTED] almost my entire adulthood over 28 years and one constant is upset stomach and achy joints." -10/2021

"When I was taking it I had horrendous insomnia and when I did sleep I had terrible nightmares. I was taken off of them due to dramatic weight loss." -10/2021

"I can't keep [REDACTED] or [REDACTED] down at all. It makes me very nauseous." -8/2021

"I'm so OVER ALL THE SIDE EFFECTS OF [REDACTED]! I hate being sick all the time. And the joint and muscle pain. I can't function." 3/2021

"This is why I stopped taking it years ago. I was sooo sick! My [REDACTED] is trying to convince me to try it again but I'm scared to." 3/2021

"after 4 months of [REDACTED] (pretty mild dose) I've lost so much weight I don't think it's worth being on it anymore. I'm sick every time I eat and haven't enjoyed a meal since before Christmas. I just think the side effects are too much for me." -3/2021

I have a lot of the side effects upset stomach, bad gas, thin skin, restless legs and severe joint pain! No pains some days are so unbearable! I've started with the buzzin' in my ears! I've been so depressed!" 3/2019

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[REDACTED] solves the problems of [REDACTED]

STRUCTURES REDACTED

FEATURE	POTENTIAL BENEFIT
[REDACTED]	<ul style="list-style-type: none">Improved compliance<ul style="list-style-type: none">Reduced GI side effects: [REDACTED]Potential to avoid food effect
Improved Bioavailability	<ul style="list-style-type: none">Less drug required for same benefit24-hour control with 1-2x/day dosing<ul style="list-style-type: none">[REDACTED] improving permeability and reducing variability in absorbanceLonger duration of circulating drug; potential 24-hour control with 1-2x/day dosing
Highly Stable	<ul style="list-style-type: none">Longer shelf life; reduced COGS<ul style="list-style-type: none">[REDACTED] highly stable
Known Regulatory Path (505b2)	<ul style="list-style-type: none">Accelerated regulatory process<ul style="list-style-type: none">Numerous examples of [REDACTED] approvalsFollow FDA guidance and reference known data

Differences Favor ATB226

FEATURE	[REDACTED]	ATB 226
Odorless	✘	✔
Stable Molecule	✘	✔
Orphan Exclusivity	✘	✔
Bioavailability	✘	EXPECTED
Formulation Potential	✘	EXPECTED
Low GI Side Effects	✘	EXPECTED
Patient Defined Outcomes in Label	✘	EXPECTED

KNOWN INFORMATION

- Human POC data published
- Animal POC data published
- AltiBio generated animal data confirms conclusions of previously published literature

Slides available under confidentiality:

- **Comparison of ATB226 to reference drug**
 - C_{max}
 - T_{max}
- **Comparison of concentration of ATB226 to reference drug in various body tissue/organs**
- **Results of AltiBio sponsored animal study**
 - Single Oral Dose Dog Plasma AUC PK Analysis
 - Single Oral Dose Dog Plasma C_{max} PK Analysis
 - Drug availability at site of action
- **Comparison of AltiBio animal study results to published literature**
- **Lifecycle Strategy**

ATB226

EXPECTED BENEFIT

- Increased stability
- Increased bioavailability
- Reduced GI side effects
- Reduced interpatient variability
- Accelerated regulatory review
 - Orphan
 - 505(b)(2)
- Seven years regulatory exclusivity

“24-Hour insurance against agony, pain, and misery.”

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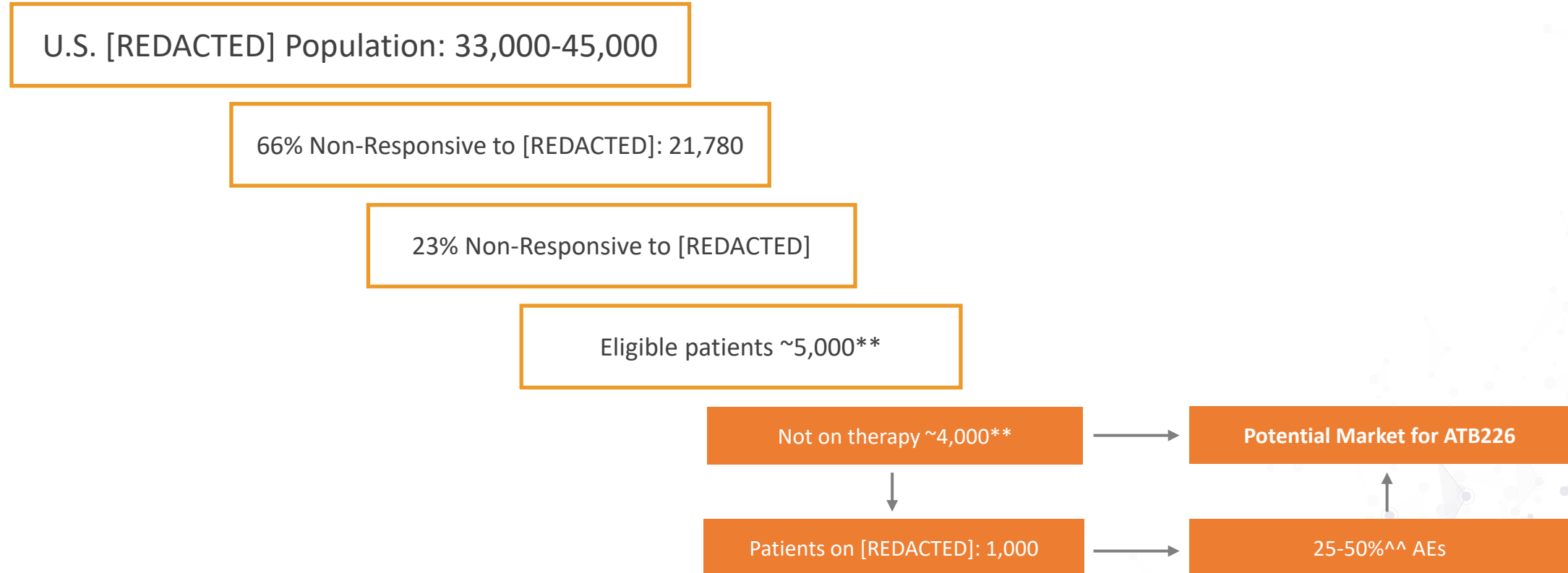
I've been in constant excruciating pain in my back, kidneys, abdomen & flanks. I have a lump under my right rib cage that hurts terribly, nausea, vomiting, not eating, lost 10 pounds in less than 2 weeks, bubbly, cloudy nasty urine, cola colored urine, not sleeping, headaches, cloudy headed, pissed off at the world & crying non-stop. I'm done...never ending UTI & kidney infections... Something HAS to be done NOW. I'm exhausted & something is very seriously WRONG & I WILL be heard & get some help,

I refuse to continue to live like this.

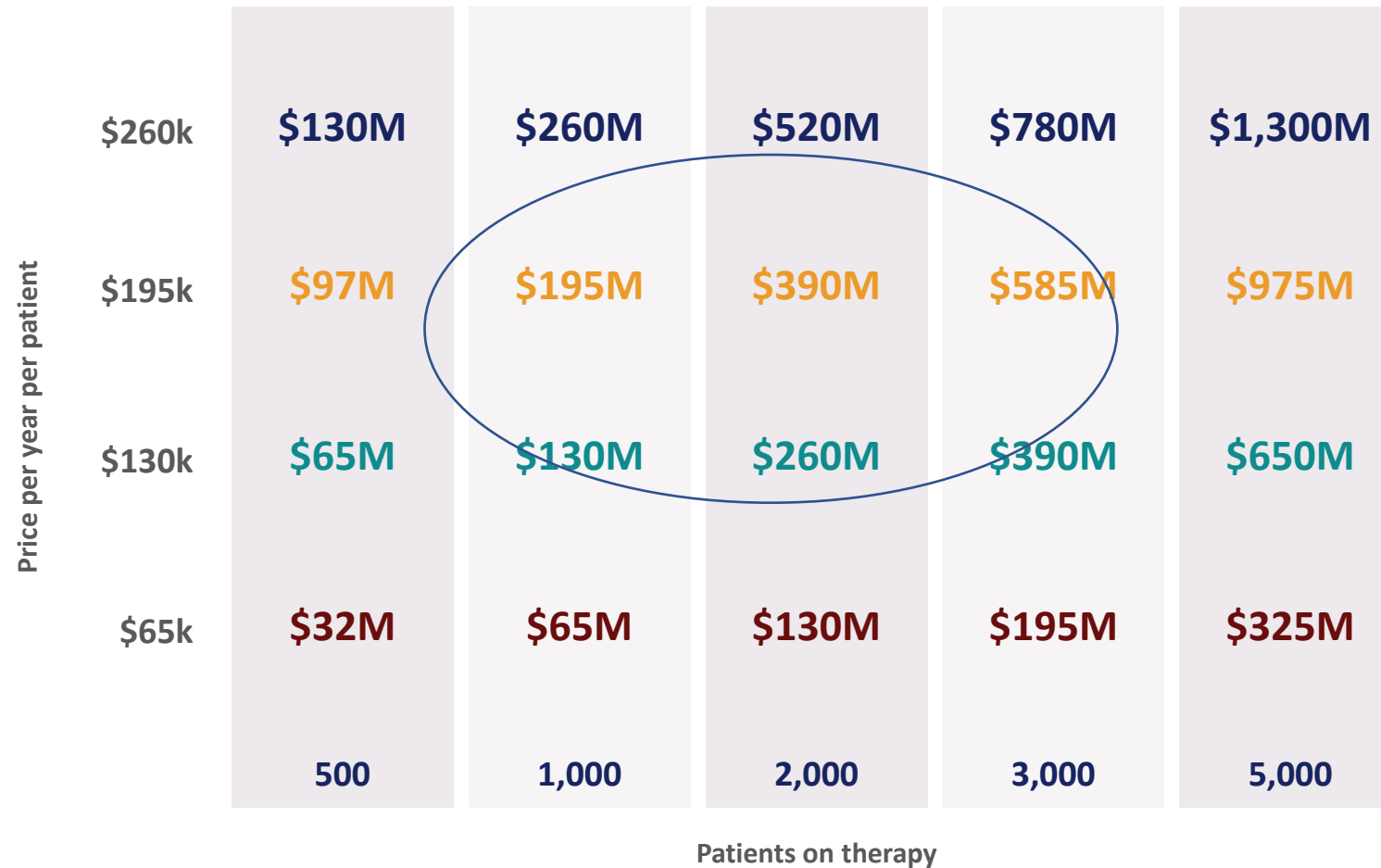
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Social media quote from patient Apr '19

Attractive Target Market for ATB226



Attractive commercial potential across a range of scenarios



Many known factors reduce relative risk in [REDACTED] development



KNOWN



KNOWN



KNOWN



KNOWN



KNOWN

NOW

STRUCTURES REDACTED

[REDACTED]

[REDACTED]
FDA-Approved

[REDACTED]
Published Human Proof of Concept



Exclusivity from:
Regulatory Patents

- Formulation
- Manufacturing



505b2 Regulatory Path
Bioequivalence,
Orphan Drug



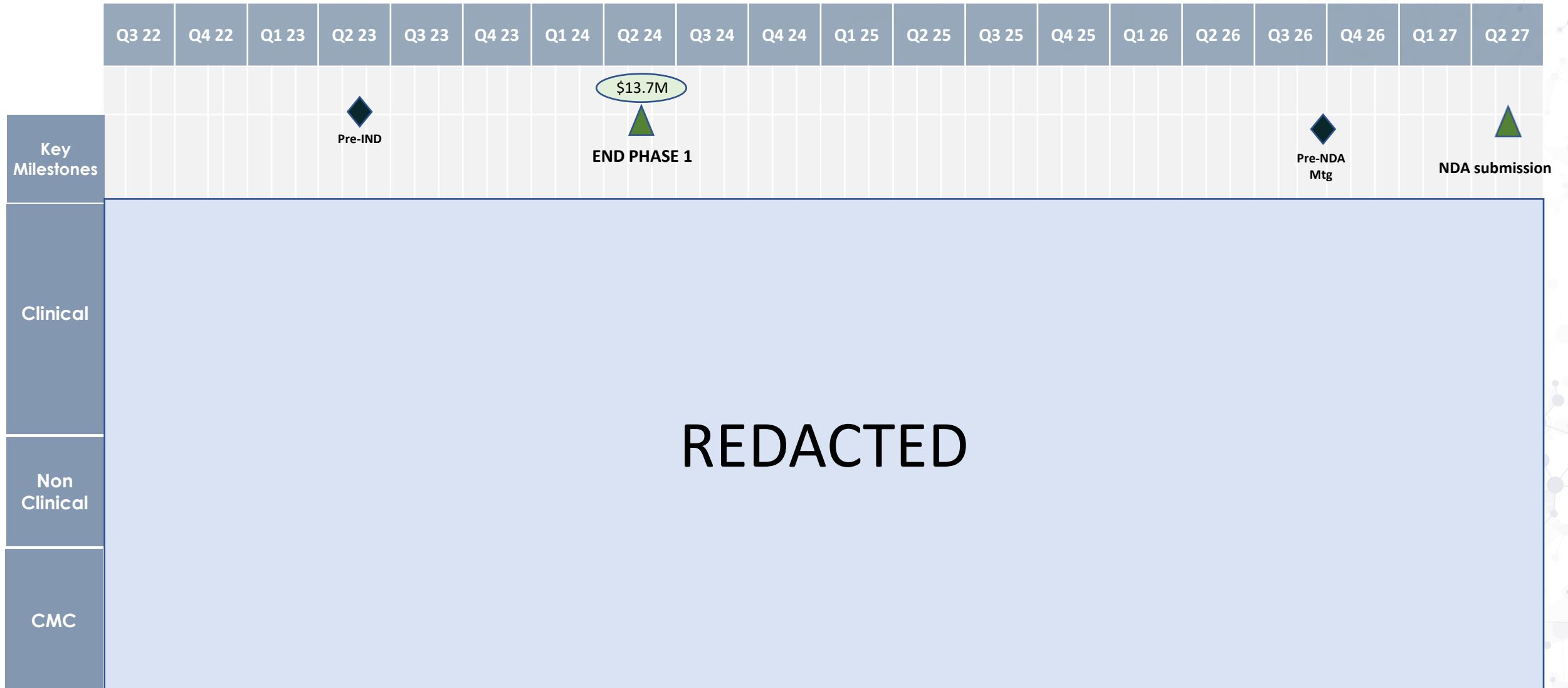
Phase 1 Healthy Volunteers
Phase 2/3 Pivotal Patients
Clinical Trial Design



DEVELOPMENT PLAN

- [REDACTED]

ATB226 Development Timeline



SOURCES & USE OF FUNDS TO COMPLETE PHASE 1

FINANCIAL PROJECTIONS

~\$15 M

Source of funds

Series B

15.0-20.0

Use of Funds

Clinical Trials

2.3

Non-clinical

3.0

CMC & Quality

6.1

Operations (Facilities, Legal, other G&A)

2.3

Total Use of Funds

13.7

AltiBio, Inc. replicating Raptor's model, but for better strategy, larger disease, stronger exclusivity...



CYSTINOSIS

500 people in US

Branded generic available

- \$8-10,000/year

Medical Need

- 24-hour control
- Dosing convenience
- Reduced pill burden
- Reduced side effects



- Orphan Drug
- 505(b)2
- 50% of patients on therapy within six months of launch
- 97% receiving payor reimbursement at \$250,000/year
- Product sales of \$100M in under 3 years
- Company raised total of \$200M prior to launch
- Company sold for \$800M, or 8x sales to Horizon Pharma



REDACTED

ATB226

- Orphan Drug
- 505(b)2
- Strong patent position
- Facilitates extended-release formulations
- REDACTED
- Wider flexibility for pricing
- Product sales potential much larger than Procysbi
- Company focus limits need for additional funds
- Company valuation basis: Multiple of annual sales? Probability of success?





**FOR MORE
INFORMATION
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OUR MISSION

*Bring relief to people living
with rare and severe diseases*

