WHAT TO WATCH FOR IN BIOTECH IN 2018

Note: The editorial content in this presentation was written and produced by the editorial staff of STAT with no participation from sponsors.
AGENDA

- A Very Brief Summary of Biotech 2017
- FDA: How Low Can the Approval Bar Go?
- Buy-side Sentiment
- Drug Pricing & Reimbursement
- Tax Reform & Repatriation
- M&A
- Biotech’s Burning Questions
- Key Clinical Trials in 2018
- 2018 Drug Launches
- IPOs
- What to Expect at #JPM18

HOSTS

- Adam Feuerstein @adamfeuerstein
  Senior Biotech Reporter
- Damian Garde @damiangarde
  National Biotech Reporter
A VERY BRIEF SUMMARY OF BIOTECH 2017
A VERY BRIEF SUMMARY OF BIOTECH 2017

• We scienced the S#@^ out of it: CAR-T, gene therapy, RNAi
• Stock Performance: Meh.

![Biotech versus the S&P 500 in 2017](source: nasdaq data)

• Trump: More bluster than bite.
• Scott Gottlieb
• IPOs in abundance
• Mixed earnings
• Fund flows: Neutral
• M&A MIA
FDA: HOW LOW CAN THE APPROVAL BAR GO?
FDA: HOW LOW CAN THE APPROVAL BAR GO?

- Scott Gottlieb is the new Oprah.
- More efficient, streamlined drug development (targeted therapies, rare diseases).
- Patient-focused drug reviews.
- Clinical data “flexibility.”
- Drug reviews to watch: Alkermes, Intra-Cellular Therapies, Sarepta Therapeutics.
- Will an “easier” FDA lead to reimbursement crackdown?
- What could go wrong? Safety!
NOVEL DRUG APPROVALS BY YEAR

Number of approvals


SOURCE: FDA DATA
## Upcoming FDA Approvals

<table>
<thead>
<tr>
<th>COMPANY</th>
<th>DRUG</th>
<th>INDICATION</th>
<th>PDUFA DATE</th>
</tr>
</thead>
<tbody>
<tr>
<td>Spark Therapeutics</td>
<td>Luxturna</td>
<td>RPE65-mediated inherited retinal dystrophies</td>
<td>January 12</td>
</tr>
<tr>
<td>Gilead Sciences</td>
<td>Bictegravir</td>
<td>HIV</td>
<td>February 12</td>
</tr>
<tr>
<td>Aerie Pharma</td>
<td>Rhopressa</td>
<td>Glaucoma</td>
<td>February 28</td>
</tr>
<tr>
<td>La Jolla Pharma</td>
<td>LJPC-501</td>
<td>Distributive shock</td>
<td>February 28</td>
</tr>
<tr>
<td>Vertex Pharma</td>
<td>Tezacaftor/Ivacaftor</td>
<td>Cystic Fibrosis</td>
<td>February 28</td>
</tr>
<tr>
<td>Novartis</td>
<td>Kymriah</td>
<td>DLBCL</td>
<td>April 30</td>
</tr>
<tr>
<td>AbbVie, Neurocrine Bioscience</td>
<td>Elagolix</td>
<td>Endometriosis</td>
<td>May 4</td>
</tr>
<tr>
<td>Amgen</td>
<td>Aimovig</td>
<td>Migraines</td>
<td>May 17</td>
</tr>
<tr>
<td>BioMarin</td>
<td>Peg-Pal</td>
<td>PKU</td>
<td>May 25</td>
</tr>
<tr>
<td>Achaogen</td>
<td>Plazomicin</td>
<td>UTI, Serious bacterial infections due to CRE</td>
<td>June 26</td>
</tr>
<tr>
<td>GW Pharma</td>
<td>Eplidiolex</td>
<td>Lennox-Gastaut, Dravet syndromes (seizures)</td>
<td>June 29</td>
</tr>
<tr>
<td>Ionis Pharma</td>
<td>Inotersen</td>
<td>TTR amyloidosis</td>
<td>July 6</td>
</tr>
<tr>
<td>Alnylam Pharma</td>
<td>Patisiran</td>
<td>TTR amyloidosis</td>
<td>August 12 (estimated)</td>
</tr>
<tr>
<td>Acorda Therapeutics</td>
<td>Inbria</td>
<td>Parkinson's</td>
<td>October 5</td>
</tr>
<tr>
<td>Alkermes</td>
<td>ALKS 5461</td>
<td>Major depressive disorder</td>
<td>TBD</td>
</tr>
<tr>
<td>Sarepta Therapeutics</td>
<td>Golodirsen</td>
<td>Exon 53 DMD</td>
<td>TBD</td>
</tr>
<tr>
<td>Avexis</td>
<td>AVXS-101</td>
<td>Spinal muscular atrophy</td>
<td>TBD</td>
</tr>
<tr>
<td>Intra-Cellular Therapies</td>
<td>Lumateperone</td>
<td>Schizophrenia</td>
<td>TBD</td>
</tr>
</tbody>
</table>
BUY-SIDE SENTIMENT: J.P. MORGAN

60% Of respondents expect the biotech sector (NBI) to outperform the broader market in 2018

11% Of respondents expect the biotech sector (NBI) to underperform the broader market in 2018

IN 2016

71% Predicted outperformance

84% Expect an uptick in M&A in 2018. In 2017, it was 93 percent.

Most important clinical trial catalyst in 2018: IDO MELANOMA DATA (INCYTE / MERCK)

SECTOR TAILWINDS
- M&A
- CLINICAL DATA/INNOVATION
- EASIER FDA REPATRIATION/TAX REFORM

SECTOR HEADWINDS
- REIMBURSEMENT/PRICING
- NEGATIVE SENTIMENT
- CLINICAL DATA FAILURE
- LACK OF M&A
BUY-SIDE SENTIMENT: RBC

THE BIOTECH SECTOR IS

11% UNDERVALUED
43% FAIRLY VALUED
43% OVERVALUED

SECTOR TAILWINDS
- M&A
- THERAPEUTIC AREA EMERGENCE
- SENTIMENT
- REGULATORY LANDSCAPE

SECTOR HEADWINDS
- DRUG PRICING/POLICY
- COMPETITION
- SENTIMENT
- PATENT CLIFFS
Do you plan to reallocate more dollars to biotech in 2018 than in 2017?

- 65% YES
- 35% NO

Are you more, less, or equally concerned about drug pricing in 2018?

- 10% MORE
- 35% LESS
- 55% EQUALLY

Do you expect biotech M&A to increase, decrease, or stay flat in 2018?

- 92% INCREASE
- 1% DECREASE
- 7% FLAT
POLL #1: SENTIMENT

Complete this sentence: In 2018, biotech stocks will _____________. 
DRUG PRICING & REIMBURSEMENT
- 2017 was all talk, no action with respect to legislative “fix” for high drug prices.

- Ex-Eli Lilly executive Alex Azar will likely run HHS.

- Insurer/PBM success stories: Hepatitis C, PCSK9s. Competitive price reductions, restricted market access.


- Orphan/rare diseases: Still protected but for how long?

- Gene/cell therapy reimbursement: outcomes-based, annuities?

- Politics: Midterm elections. Renewed bipartisan push to enact drug-price legislation?

- Biosimilars: They’re here, not going away.
TAX REFORM &
REPATRIATION
Biotech companies already carry low tax rates because of multinational operations.

Repatriation of overseas profits will have larger impact.

Amgen: $40B in overseas cash.

Gilead: $31B in overseas cash.

M&A driver or more share buybacks?
Thesis remains same: Large-cap biotech suffering from slowing growth due to maturing drug franchises and relative paucity of late-stage pipeline assets.

Ready access to cash and debt.

Hence: More M&A.

2017 was disappointing.

9 public M&A deals in 2017 = $57B compared to 10 public M&A deals in 2016 = $59B

LARGEST 2017 DEALS

- Johnson & Johnson for Actelion $30B
- Gilead for Kite Pharma $11B

HOTTEST THERAPEUTICS CATEGORIES REMAIN THE SAME

- Oncology
- CNS/neuro-psych
- Rare disease

TOP M&A CANDIDATES

Note: The usual suspects.
POLL #2: M&A

Which one is most likely to be acquired?
ADAM’S TAKE: BIOTECH’S BURNING QUESTIONS
Can they restore investor confidence and find new growth drivers following the mongersen failure and 2020 guidance cut?

Can they execute on the bictegravir and Yescarta launches? Can they stabilize hepatitis C sales?

Will an Alzheimer’s drug finally work? If not, does Biogen have a Plan B?

What happens if ALXN1210 fails?

How fast can the cystic fibrosis triple combos be approved?
ADAM’S TAKE: BIOTECH’S BURNING QUESTIONS

**Incyte**
Epacodostat better work.
If not…?

**BIOMARIN**
When will you stop losing money?

**AMGEN**
Will you use all those billions of dollars on something other than paying your lawyers?

**REGENERON**
Will the pace of the Dupixent launch continue to grow? Is there anything truly innovative in the pipeline?
KEY CLINICAL TRIALS TO WATCH
## Key Clinical Trials to Watch

<table>
<thead>
<tr>
<th>COMPANY</th>
<th>DRUG</th>
<th>INDICATION</th>
<th>ESTIMATED DATA READOUT</th>
</tr>
</thead>
<tbody>
<tr>
<td>Dermira</td>
<td>DRM01 (Olumacostat Glasaretil)</td>
<td>Acne</td>
<td>Q1</td>
</tr>
<tr>
<td>Aimmune</td>
<td>AR101</td>
<td>Peanut allergy</td>
<td>Q1</td>
</tr>
<tr>
<td>Incyte, Merck</td>
<td>Epacadostat+Keytruda</td>
<td>Melanoma</td>
<td>1H</td>
</tr>
<tr>
<td>AstraZeneca</td>
<td>Imfinzi+treme</td>
<td>Lung cancer, head &amp; neck cancer</td>
<td>1H</td>
</tr>
<tr>
<td>Esperion</td>
<td>ETC-1002 (bempedoic acid)</td>
<td>LDL-C lowering</td>
<td>Q2</td>
</tr>
<tr>
<td>Alexion Pharma</td>
<td>ALXN1210</td>
<td>PNH</td>
<td>Q2</td>
</tr>
<tr>
<td>Acceleron, Celgene</td>
<td>Luspatercept</td>
<td>MDS, beta-thalassemia</td>
<td>Q2</td>
</tr>
<tr>
<td>Celldex</td>
<td>Glembatumumab</td>
<td>Triple-negative breast cancer</td>
<td>Q2</td>
</tr>
<tr>
<td>Dicerna</td>
<td>DCR-PHXC</td>
<td>Primary Hyperoxaluria</td>
<td>2H</td>
</tr>
<tr>
<td>Juno Therapeutics</td>
<td>JCAR017</td>
<td>DLBCL</td>
<td>2H</td>
</tr>
</tbody>
</table>
2018 DRUG LAUNCHES
## Important 2018 Drug Launches

<table>
<thead>
<tr>
<th>DRUG</th>
<th>COMPANY</th>
<th>LAUNCH DATE</th>
<th>Consensus Sales Estimate – 2018</th>
<th>Consensus Sales Estimate – 2022</th>
</tr>
</thead>
<tbody>
<tr>
<td>Yescarta</td>
<td>Gilead Sciences</td>
<td>Q4 17</td>
<td>$209M</td>
<td>$1.39B</td>
</tr>
<tr>
<td>Kymriah</td>
<td>Novartis</td>
<td>Q4 17</td>
<td>$116M</td>
<td>$992M</td>
</tr>
<tr>
<td>Repatha</td>
<td>Amgen</td>
<td>2H 15</td>
<td>$784M</td>
<td>$2.71B</td>
</tr>
<tr>
<td>Praluent</td>
<td>Regeneron/Sanofi</td>
<td>2H 15</td>
<td>$295M</td>
<td>$1.77B</td>
</tr>
<tr>
<td>Luxturna</td>
<td>Spark</td>
<td>Q1 18</td>
<td>$75M</td>
<td>$364M</td>
</tr>
<tr>
<td>Bictegravir</td>
<td>Gilead Sciences</td>
<td>Q1 18</td>
<td>$884M</td>
<td>$5.05B</td>
</tr>
<tr>
<td>Inotersen</td>
<td>Ionis Pharma</td>
<td>Q3 18</td>
<td>$5M</td>
<td>$436M</td>
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<tr>
<td>Patisiran</td>
<td>Alnylam/Sanofi</td>
<td>Q3 18</td>
<td>$48.5M</td>
<td>$1.02B</td>
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<tr>
<td>Aimovig</td>
<td>Amgen/Novartis</td>
<td>Q2 18</td>
<td>$92M</td>
<td>$1.22B</td>
</tr>
<tr>
<td>Fremanezumab</td>
<td>Teva</td>
<td>2H 18</td>
<td>TBD</td>
<td>$607M</td>
</tr>
</tbody>
</table>

SOURCE: Evaluate
IPO MARKET
2017 was a solid year: 40 IPOs and the best average return since 2013

But can it last?

Biotech didn't beat the S&P 500 in 2017, and fund flows were negative on the year

And can the unicorns maintain their valuations?

Source: NASDAQ data
J.P. MORGAN EXPECTATIONS
POLL #3: JPM EXPECTATIONS

Which of these things is most likely to happen at #JPM18?
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