Defined Health is pleased to present:

26th Annual Cancer Progress Conference  
March, 2015  
New York City, NY  
www.cancerprogressbyDH.com

BioEurope Spring  
March 9-11, 2015  
Paris, France  
www.therapeuticinsight.com

Defined Health will also be participating in the following industry events:


26th Annual Cancer Progress Conference  
March, 2015  
New York City, NY  
www.cancerprogressbyDH.com

BioEurope Spring  
March 9-11, 2015  
Paris, France  
www.therapeuticinsight.com

Defined Health will also be participating in the following industry events:

Establishing Proof of Relevance for Early Stage Deal Making

Ed Saltzman
President
Defined Health

Webinar
April 2014
Longstanding Conventional Wisdom About PoC-Driven Value Inflection

“Today, the big inflection points are:

i. initial P2 results, which suggest efficacy

ii. advanced P3 trials, which lead to a New Drug Application (NDA), and

iii. market launch.”

Source: Nature Biotechnology 20, BE36-BE39 (June 2002)

Based on: Burrill & Co., and Ernst & Young, 1997

http://www.nature.com/bioent/building/planning/012003/fig_tab/nbt0602supp-BE36_F2.html
Needs Reconsideration
# Large Pre-PoC Deals, Once Rare, Became Common in 2012

<table>
<thead>
<tr>
<th>Licensor</th>
<th>Partner</th>
<th>Product / Technology - 2012 DEALS</th>
<th>Headline $m</th>
<th>Upfront ( $m) / % of headline</th>
</tr>
</thead>
<tbody>
<tr>
<td>Molecular Partners</td>
<td>Allergan</td>
<td>MP0260 PC dual anti VEGF-A/PDGFB-DARPins for wet AMD; discovery alliance/options against selected targets in serious eye disease</td>
<td>1,463</td>
<td>62.5 / 4.3%</td>
</tr>
<tr>
<td>Galapagos</td>
<td>Abbott</td>
<td>Collaboration for oral JAK1 inhibitor (GLPG0634) in RA and other autoimmune diseases (P2)</td>
<td>1,350</td>
<td>150 / 11.1%</td>
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<tr>
<td>Five Prime Tx</td>
<td>GSK</td>
<td>Discovery collaboration with options for asthma and COPD targets</td>
<td>1,191</td>
<td>30 over 4y / 2.5%</td>
</tr>
<tr>
<td>Genmab</td>
<td>J&amp;J</td>
<td>License to daratumumab, oncology (P1/2)</td>
<td>1,100</td>
<td>135 / 12.3%</td>
</tr>
<tr>
<td>MacroGenics</td>
<td>Servier</td>
<td>Discovery alliance with options for Dual-Affinity Re-Targeting (DART) platform technology for 3 cancer targets</td>
<td>1,100</td>
<td>20 / 1.8%</td>
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<tr>
<td>Endocyte</td>
<td>Merck</td>
<td>License to vintafolide for ovarian cancer, NSCLC and other solid tumors (P3)</td>
<td>1,000</td>
<td>120 / 12%</td>
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<tr>
<td>Astellas</td>
<td>J&amp;J</td>
<td>ex-JPN rights to ASP015K, an oral JAK inhibitor for RA and psoriasis (P2)</td>
<td>945</td>
<td>65 / 6.9%</td>
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<tr>
<td>Selecta</td>
<td>Sanofi</td>
<td>Discovery collaboration for antigen-specific immuno-therapies for life threatening allergies based on Synthetic Vaccine Particle platform</td>
<td>900</td>
<td>ND</td>
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<tr>
<td>Forma Tx</td>
<td>Boehringer Ingelheim</td>
<td>Discovery of small molecule therapeutics against oncology-relevant protein-protein interactions</td>
<td>815</td>
<td>65 / 8%</td>
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<tr>
<td>Evotec</td>
<td>Bayer</td>
<td>5 year multi-target collaboration to develop 3 candidates for endometriosis</td>
<td>765</td>
<td>15 / 2%</td>
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<tr>
<td>Forma Tx</td>
<td>J&amp;J</td>
<td>Discovery/dev - novel small molecule candidates - target tumor metabolism</td>
<td>700</td>
<td>ND</td>
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<tr>
<td>Xenon</td>
<td>Genentech</td>
<td>Strategic alliance to discover, develop drugs and companion Dx for pain</td>
<td>646</td>
<td>ND</td>
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<tr>
<td>Symphogen</td>
<td>Merck KgaA</td>
<td>License to Sym004 - antibody targeting EGFR in advanced metastatic CRC (P1/2) and squamous cell carcinoma head and neck (P2)</td>
<td>638</td>
<td>26 / 4.1%</td>
</tr>
<tr>
<td>Isis Pharma</td>
<td>Biogen Idec</td>
<td>License with options to discover/develop antisense drugs against 3 undisclosed targets to treat neurological disorders</td>
<td>630</td>
<td>30 / 4.8%</td>
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<tr>
<td>Ablynx</td>
<td>Merck</td>
<td>License for discovery of Nanobodies at a voltage gated ion channel target</td>
<td>587</td>
<td>8 / 1.4%</td>
</tr>
</tbody>
</table>
And Continued Through 2013

Top 16 Licensing Deals in 2013

<table>
<thead>
<tr>
<th>Company</th>
<th>Deal Partner</th>
<th>Product</th>
<th>Status on Deal Date (2013)</th>
<th>Deal Value ($m)</th>
<th>Upfront Payment ($m)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Dainippon Sumitomo</td>
<td>Edison</td>
<td>EPI-589</td>
<td>Research project</td>
<td>4,238</td>
<td>18</td>
</tr>
<tr>
<td>Biogen Idec</td>
<td>Elan</td>
<td>Tysabri</td>
<td>Marketed</td>
<td>3,250</td>
<td>3,250</td>
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<tr>
<td>Lilly</td>
<td>Pfizer</td>
<td>Tanezumab</td>
<td>Phase III</td>
<td>1,780</td>
<td>200</td>
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<tr>
<td>Roche</td>
<td>Molecular Partners</td>
<td>Cancer Program</td>
<td>Research project</td>
<td>1,156</td>
<td>60</td>
</tr>
<tr>
<td>Roche</td>
<td>immatics biotechnologies</td>
<td>IMA942</td>
<td>Pre-clinical</td>
<td>1,017</td>
<td>17</td>
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<tr>
<td>Celgene</td>
<td>Oncomed</td>
<td>Demcizumab</td>
<td>Phase II</td>
<td>967</td>
<td>155</td>
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<tr>
<td>Celgene</td>
<td>MorphoSys</td>
<td>MOR202</td>
<td>Phase II</td>
<td>910</td>
<td>92</td>
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<tr>
<td>Gilead</td>
<td>MacroGenics</td>
<td>DART Program 1</td>
<td>Research project</td>
<td>836</td>
<td>23</td>
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<tr>
<td>Otsuka</td>
<td>Lundbeck</td>
<td>Lu AE58054</td>
<td>Phase II</td>
<td>825</td>
<td>150</td>
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<tr>
<td>AstraZeneca</td>
<td>FibroGen</td>
<td>Roxadustat</td>
<td>Phase III</td>
<td>815</td>
<td>350</td>
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<tr>
<td>AbbVie</td>
<td>Ablynx</td>
<td>ALX-0061</td>
<td>Phase II</td>
<td>665</td>
<td>-</td>
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<tr>
<td>Roche</td>
<td>Isis</td>
<td>Huntington's Disease Project</td>
<td>Research project</td>
<td>659</td>
<td>30</td>
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<tr>
<td>Pfizer</td>
<td>CytomX Therapeutics</td>
<td>Cancer Antibody Research</td>
<td>Research project</td>
<td>635</td>
<td>25</td>
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<tr>
<td>Roche</td>
<td>Chiasma</td>
<td>Octreolin</td>
<td>Phase III</td>
<td>595</td>
<td>65</td>
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<tr>
<td>Roche</td>
<td>Prothena</td>
<td>PRX002</td>
<td>Pre-clinical</td>
<td>585</td>
<td>30</td>
</tr>
<tr>
<td>AstraZeneca</td>
<td>Moderna</td>
<td>mRNA therapeutics – cancer</td>
<td>Discovery</td>
<td>CONF</td>
<td>240</td>
</tr>
</tbody>
</table>
Which Defined Health Predicted a Few Years Ago

However, Pharma’s R&D Recalibration Means They Will Need to Feed Early Pipelines Externally

- Biggest internal cuts are in discovery so IND-ready P2 pipeline will need to be filled externally, especially as current internal programs are being jettisoned

- Biotechs’ inventory of P2b programs is not being replenished and Pharma has already picked this tree pretty clean

- Pharma will need to accept increased scientific/regulatory risk in order to fill early pipelines - though there will be “negotiation tension” around size of upfront commitment for pre PoC programs

- But Pharma’s R&D strategy recalibration means there will zero tolerance for commercial risk and a heightened wariness of downstream regulatory risk
AstraZeneca details R&D overhaul

March 2, 2010

London--AstraZeneca plc said Tuesday it will stop research into several diseases and close sites in the US and Europe. The move is part of an overhaul of the drug maker's research-and-development operations which it first announced at the end of January.

AstraZeneca said Tuesday it was ending discovery research in thrombosis, acid reflux, ovarian and bladder cancers, systemic scleroderma, schizophrenia, bipolar disorder, depression and anxiety, hepatitis C and vaccines other than respiratory syncytial virus and influenza.

Early-stage discovery research will cease at the company's U.S. facility in Wilmington, Del., and it will close its facility in Lund, Sweden. In the U.K., Charnwood and a smaller facility, acquired with Kudos, in Cambridge will be shuttered. Pharmaceutical development work at the Avon facility will also end, and AstraZeneca said it aims to sell its arrow therapeutics business in London.

On Jan. 28, AstraZeneca said it would build on previous restructuring efforts by revamping its R&D operations--a move that cuts 8,000 jobs across the company, including a net reduction of 1,800 in R&D.

On Jan. 28, AstraZeneca said it would build on previous restructuring efforts by revamping its R&D operations--a move that cuts 8,000 jobs across the company, including a net reduction of 1,800 in R&D. AstraZeneca, the U.K.'s second largest drugmaker by sales after GSK, was formed in 1999 by the merger of Sweden's Astra AB and U.K.-based Zeneca group. It currently has more than 65,000 employees and operations in more than 100 countries, according to its web site.
Pfizer Plans to Cut Research Spending By Up To $3B

February 4, 2010

Pfizer Inc. said Wednesday that it plans to cut research-and-development spending by as much as $3 billion by 2012, in an attempt to wring efficiencies following its take-over of Wyeth without sacrificing future product development. The New York pharmaceutical giant outlined the aggressive cuts, which represent more than a quarter of the two companies’ combined research budgets in 2008, as it reported fourth-quarter earnings of $767 million, or 10 cents a share, up from $266 million, or four cents a share, a year earlier. The drug maker also forecast lower financial targets from the Wyeth deal than it provided last year, sending Pfizer shares down 2.3% to $18.62 on the New York Stock Exchange.

Research is considered the lifeblood of pharmaceutical companies. Big drug makers like Eli Lilly & Co. and Bristol-Myers Squibb Co. are increasing their spending to find new products that can replace aging blockbusters. Yet drug discovery is unpredictable, and industry scientists have struggled in coming up with big new products. Pfizer’s announcement suggests executives believe its research hasn’t been worth the high levels of investment.

The largest drug makers have been retrenching as blockbuster drugs start facing competition from less expensive generic versions. After early cost cutting focused on trimming sales representatives, companies have set their sights on labs and scientists. Last week, AstraZeneca PLC said it would cut about 3,500 research jobs as part of a larger workforce reduction. Pfizer also targeted research previously, cutting as many as 800 research workers last year and closing six of 20 research sites as part of the Wyeth acquisition completed in October. Meantime, the $68 billion Wyeth takeover and other deals are filling Pfizer’s pipeline with therapies discovered elsewhere.

Chief Executive Jeffrey Kindler said he believes Pfizer has struck the “right balance” between efficiency and innovation by embracing deals and partnerships while still investing heavily in internal research. "The days of a monolithic approach to either research or commercialization are behind us," he said. Pfizer said its research expenses would be $8 billion to $8.5 billion in 2012. The combined research spending for the two companies was $11 billion in 2008, the last full year before the merger. Chief Financial Officer Frank D’Amelio said the research cuts are aimed to reduce overlaps in the two companies. The planned cuts are part of a total of $7 billion in savings Pfizer expects to realize from both the Wyeth deal and its cost-cutting efforts begun before the acquisition. Pfizer will still have among the industry’s biggest research budgets, company officials said. Chief executives at GlaxoSmithKline PLC and Sanofi-Aventis SA have talked about the poor returns from company labs and reworking their approach to drug development. Sanofi Chief Financial Officer Jerome Contamine said in July that the company planned to cut R&D spending by 20% between 2008 and 2011. Consultants like Terry Hisey, vice chairman and U.S. life sciences leader at Deloitte LLP, is telling drug makers that it is "more cost effective" to bring in promising therapies from the outside than trying to develop them all in-house. Yet Sanford Bernstein analyst Tim Anderson said the research cuts by drug conglomerates could "come back to bite" the companies because they will need new products.

It Seemed Inevitable That Early Stage Pipelines Would Shrink

Number of Pre-Clinical and Phase 1 Programs in Top 10 Pharma Pipelines (2003-2013)

- Phase 1
- Pre-Clinical

EvaluatePharma
But *Decline* in Clinical Phase Deal Values Not Easily Predictable

# Phase 1 to Phase 3 Product Deal by Therapy Area

<table>
<thead>
<tr>
<th>Therapy Area</th>
<th>Product deal count</th>
<th>Up-front payments ($m)</th>
<th>Total deal value ($m)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Oncology &amp; Immunomods</strong></td>
<td>51</td>
<td>93</td>
<td>51</td>
</tr>
<tr>
<td><strong>Systemic anti-infectives</strong></td>
<td>25</td>
<td>34</td>
<td>33</td>
</tr>
<tr>
<td><strong>Central nervous system</strong></td>
<td>42</td>
<td>49</td>
<td>22</td>
</tr>
<tr>
<td><strong>Gastro-intestinal</strong></td>
<td>19</td>
<td>21</td>
<td>13</td>
</tr>
<tr>
<td><strong>Respiratory</strong></td>
<td>13</td>
<td>14</td>
<td>11</td>
</tr>
<tr>
<td><strong>Musculoskeletal</strong></td>
<td>6</td>
<td>13</td>
<td>10</td>
</tr>
<tr>
<td><strong>Endocrine</strong></td>
<td>8</td>
<td>14</td>
<td>8</td>
</tr>
<tr>
<td><strong>Cardiovascular</strong></td>
<td>17</td>
<td>16</td>
<td>7</td>
</tr>
<tr>
<td><strong>Dermatology</strong></td>
<td>12</td>
<td>5</td>
<td>6</td>
</tr>
<tr>
<td><strong>Various</strong></td>
<td>8</td>
<td>6</td>
<td>6</td>
</tr>
<tr>
<td><strong>Blood</strong></td>
<td>2</td>
<td>7</td>
<td>4</td>
</tr>
<tr>
<td><strong>Genito-urinary</strong></td>
<td>17</td>
<td>7</td>
<td>3</td>
</tr>
<tr>
<td><strong>Sensory organs</strong></td>
<td>7</td>
<td>10</td>
<td>1</td>
</tr>
<tr>
<td><strong>Totals</strong></td>
<td>233</td>
<td>289</td>
<td>175</td>
</tr>
</tbody>
</table>

* CNS increase: Lilly/Pfizer deal for Tanezumab; $200M upfront, $1,780M total deal value
* CV increase: Forest/Trevana deal for AT-1 antag; $460M total deal value
* Blood increase: AZN/Fibrogen deal for Roxadustat; $400M upfront, $815M total deal value

EvaluatePharma report - Therapy area licensing in 2012 – Rags and riches

PoR Webinar, April 2014
© Defined Health
Or Explainable...

Big Pharma Sales CAGR (2011-2018)

Top Pharma Sales CAGRs (2011-2018)

Source: Pharmaview
Supply of “Available” Clinical Stage Programs Remains Healthy

Number of Unpartnered Compounds Owned by Non-Commercial Stage Biotechs

- Phase 3
- Phase 2
- Phase 1

Data analysis provided courtesy of Thomson Pharma IDdb
But Still Far Exceeds Demand

Deals as a Percentage of Biotech Programs by Phase

- Phase 2
- Phase 1

EvaluatePharma, Thomson Reuters
Much Low Hanging “post PoC Fruit” Already Picked Clean

Abbott and Galapagos Announce Global Collaboration for Novel Oral Therapy, GLPG0634, in Phase II to Treat Autoimmune Diseases
29 February 2012

J&J nabs Pharmacyclics cancer candidate in $975M deal
December 9, 2011

Some Fundamentals Never Change

### Estimates of the Full Cost of Bringing an NME to Market

<table>
<thead>
<tr>
<th>Source</th>
<th>US$M, 2011 prices</th>
</tr>
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<tr>
<td>Hansen, 1979</td>
<td>$199 M</td>
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<tr>
<td>Wiggins, 1987</td>
<td>$226 M</td>
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<tr>
<td>DiMasi et al, 1991</td>
<td>$451 M</td>
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<tr>
<td>OTA, 1993</td>
<td>$625 M</td>
</tr>
<tr>
<td>Myers and Howe, 1997</td>
<td>$664 M</td>
</tr>
<tr>
<td>DiMasi et al, 2003</td>
<td>$1,031 M</td>
</tr>
<tr>
<td>Adams and Branter, 2006</td>
<td>$1,116 M</td>
</tr>
<tr>
<td>Adams and Branter, 2010</td>
<td>$1,560 M</td>
</tr>
<tr>
<td>Paul et al, 2010</td>
<td>$1,867 M</td>
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<tr>
<td>Mester-Ferrandiz et al, 2012</td>
<td>$1,506 M</td>
</tr>
</tbody>
</table>

Parexel Biopharmaceutical R&D Statistical Handbook, compiled from various sources listed
Except This One!
Commercial Risk Already Off-the-Charts Will Only Get Worse

10 top drug launch disasters

Payer risk looms large as biopharma marketing snafus highlight industry's Achilles' heel

November 27, 2012

http://www.fiercepharma.com/special-reports/top-10-drug-launch-disasters
Pharma Clearing Out Products That Cannot Be Sold

Reported Reasons for Drug Discontinuations 2003-2011

Reported Reason

Citeline analytics, Parexel Biopharmaceutical R&D Statistical Sourcebook 2013/2014, total reports each year range from 99-209, only drugs with actual declared reasons are included.
“Sweet Spot” for Program Value Inflection is When Both of These are De-Risked
NPV Greatest When Scientific and Commercial Risk are Balanced
But Magnitude of Today’s Commercial Hurdles Means Pharma More Accepting of Scientific Risk
But Only if Eventual Commercial TPP is Clear and Compelling

Which is possible to achieve prior to PoC
Perceived Commercial Risk Now Most Significant Cause of Value Destruction
Which Means for First Time, Significant Value Inflection is Now Possible Pre-PoC

http://www.nature.com/bioent/building/planning/012003/fig_tab/nbt0602supp-BE36_F2.html; modified by Defined Health
LICENSING 2013: DEAL VOLUME AND DEAL VALUE BY STAGE

License Deals By Stage At Signing: 2013

- Chart excludes 325 license deals in 2013 for which a stage at signing was unknown or not applicable.
- Note that the Celgene/Oncomed has been removed from the chart as the $3+B deal skewed Phase I deal values substantially.
LICENSING 2013: MEDIAN UPFRONT PAYMENTS BY STAGE

A seller’s market for Discovery and Phase II assets in 2013

Median Upfront Cash By Stage For Product and Technology Licenses (2008-2012 vs. 2013)

n= 405 for 2008-2012 and n = 48 for 2013 product and technology licenses (Discovery-Registration stage) with disclosed upfront cash payments

*2013 deals revealed no Registration Stage deals with disclosed upfront deal values
Proof of Relevance™
“Risky” Programs in Clinic Now Inflect Value Pre-PoC

Trevena Lands $430M Forest Deal, $60M Venture Round as Heart Drug Heads to PhIIb

May 9, 2013

With its lead drug poised to begin a Phase IIb study for acute heart failure, Trevena grabbed a $30 million investment from Forest Laboratories, which also picked up an option to snag global rights to the biotech’s lead therapy with a deal that includes a $430 million potential payout. Forest will now step in to jointly oversee the next trial for TRV027 and Trevena banks a $60 million C series with the help of Forest and its existing venture syndicate.

King of Prussia, PA-based Trevena has been working with a GPCR biased ligand drug platform. Their lead therapy is an intravenous treatment for acute decompensated heart failure (ADHF). CEO Maxine Gowen tells FierceBiotech that she's not free to divulge all the details of the Forest deal, declining to say whether an upfront payment was included in the package. But she does have enough cash in the bank now for the Phase IIb, along with some backup work on three other projects and a partner in position to step in if the data points to Phase III.

"We’ve been talking to Forest now for a couple of years," says Gowen, which expressed some keen interest in acute heart failure. "We started to really turn up the volume on the conversation when we got our Phase IIa data toward the end of last year. At the same time, we were looking to raise new cash. As these two things were going along in parallel, we had a very good conversation, with both of us saying what we mean to do. Through that we came up with this structure."

New Enterprise Associates, Polaris Venture Partners, Alta Partners, Healthcare Ventures and Yasuda Economic Development Corporation all chipped in to this round and backed a $35 million round three years ago.

Trevena’s platform technology has the potential to hit a variety of targets. And Gowen says that behind 027 there are three other programs, including another hospital-focused IV therapy for post-operative pain and a follow-on oral treatment. "Our ideal situation would be to partner that oral follow-on," says Gowen. "It’s much more relevant to the primary care market."

Gowen’s not exactly sure where the company will get its next package of funding, but with the market showing signs of warmth to biotechs, Trevena could go public--provided the market conditions stay favorable.

"ADHF is the fourth leading cause of hospitalizations in the United States and there has been no material change in the standard of care for patients with ADHF for decades," said David Solomon, Forest's SVP of corporate development and strategic planning, in a statement. "TRV027 has the potential to be a significant new advance in the treatment of ADHF because it addresses the underlying pathophysiology of the disease which has been demonstrated in pre-clinical and early clinical work by Trevena."

Roche Heralds Its Return to the Antibiotics Field with $560M Polyphor Deal

November 4, 2013

With healthcare officials around the world ringing alarm bells over a weak global pipeline of antibiotics, Roche's pRED group is making good on its promised return to the field with a $560 million development deal, partnering up with a Phase II program underway at Polyphor. In the deal, Switzerland's Polyphor--which has been advancing a new group of treatments using macrocycle technology--gets a $40 million upfront and up to $520 million for a range of milestones covering development and commercialization work. Polyphor also stands to receive a double-digit royalty stream.

"As the incidence of drug-resistant infections is creating an urgent demand for new therapeutic options, we look forward to adding this potentially important, targeted agent with a novel mechanism of action to our portfolio of innovative medicines," noted Janet Hammond, head of infectious diseases discovery and translational area in Roche's pRED.

Roche abandoned antibiotics back in 1999 as Big Pharma was beginning its retreat from an R&D arena that became known as a market loser for big players. The exodus included a number of exits and restructurings over the years, including Pfizer's decision to shut down its infectious diseases unit in Groton, CT, in 2011 and ship the research work to China. But John Reed, Roche's new head of the Basel-based pRED group, signaled to FierceBiotech recently that the pharma giant was interested in striking new deals for antibiotics that could fight bacteria found in hospitals, where antibiotics offered a more attractive proposition for developers.

Roche's return to antibiotics is focused on POL7080, a Phase II antibiotic that fights infections spurred by hospital-acquired Pseudomonas aeruginosa. Polyphor has been working on macrocycles, a new class of synthetically engineered ring-shaped drugs which are larger than traditional small molecules, with more of the potency found in large-molecule biologics. Ensemble Therapeutics, for example, has been building a library of macrocycles that can address protein-protein and protein-peptide interactions.

"As the incidence of drug-resistant infections is creating an urgent demand for new therapeutic options, we look forward to adding this potentially important, targeted agent with a novel mechanism of action to our portfolio of innovative medicines," noted Janet Hammond, head of infectious diseases discovery and translational area in Roche's pRED.

Roche's R&D arm is divided between pRED and gRED, which encompasses Genentech's work. Roche's pRED has undergone a major restructuring capped with the closure of its old R&D campus in Nutley, NJ, and the creation of a new unit in Manhattan.

But Only if Commercial Risk Looks Insignificant

2013 Alliance of the Year Nominee: Roche/Polyphor

Friday, December 13, 2013

If catching an early big wave together justifies an alliance nomination then Roche and Polyphor look like contenders. They're hoping to get a long, lucrative ride using novel science and new US and European regulations aimed at speeding up antibiotic development and approval. And by choosing Switzerland-based Polyphor’s investigational macrocycle antibiotic POL7080 for development and commercialization, Roche has given notice that it’s back in the antimicrobials space again. For the first time in 30 years. In other words Roche hasn't been interested in developing antibiotics since most of you were old enough to vote -- a development worth honoring with a DOTY nod. The pact gives micropharma Polyphor a partner to help it punch above its weight. Its three drug candidates, developed using its protein mimic technology, are POL7080; POL6326, a CXCR4 antagonist currently in Phase II and ear...
Pfizer has stepped in with an upfront check for $25 million to license in rights to an experimental, preclinical program aimed at replacing pooled IVIG therapy for autoimmune diseases. Currently, patients with autoimmune diseases get transfusions of human intravenous immunoglobulin to bolster their antibody defense system, essential in repelling diseases. But Baltimore-based Gliknik's lead program for GL-2045 is making a bid to do IVIG one better.

"GL-2045 is the first of several innovative drug candidates Gliknik is advancing for people with autoimmune diseases and cancer," said Gliknik CEO David Block in a statement. "We selected Pfizer as our partner to progress GL-2045 from among several interested and capable parties because of its exceptional development, manufacturing and commercial capabilities."

Pfizer also has the deep pockets necessary to spend $25 million for an upfront on a drug like this. The deal is tied to a slate of milestones, which were not revealed today. But the pact does illustrate Pfizer's appetite for new deals since it completed a major restructuring -- and downsizing -- of its R&D group.

Gliknik's big idea here is that its recombinant Fc fusion protein can be used in much smaller quantities to provide the same or better therapeutic effect than IVIG has. Block tells FierceBiotech that transfusions now can take two days. This new program could potentially offer a major improvement in the lives of patients with autoimmune diseases. The commercial case is also compelling. By steering clear of using human blood in the manufacturing process, Pfizer could be in a position to make an unlimited quantity to replace what is today a $7 billion product which is in high demand.

The sudden change in fortune is dramatic for the semivirtual Gliknik, which has "five-and-a-half employees," according to Block. The CEO has had to execute 9 different fundraisings since the company was founded in the dark days of 2007. Now Gliknik will have enough money to turn to its follow-up programs -- two midstage immune modulators as well as other Stradomers from the platform technology that don't compete directly with Pfizer's new program -- without having to spend time raising more cash for a while.

Gliknik has raised a total of $22.8 million in venture cash and about $3 million in contracts and grants, says Block, with $12 million of that still in the thrifty group's bank account. And the state of Maryland has come through with some essential support through its biotechnology tax credit program.

"Were it not for that program I'm not sure we would have made it to this day," notes Block in an e-mail. "For qualified biotech companies in the state of Maryland, investors from any state in the country or even the world receive back 50% of their investment with limitations at the individual, company, and state level. Fabulous program that Governor O'Malley has promoted even through budget shortfalls."

Pfizer is benefiting from Gliknik's survival: "Pooled IVIG has a long history of use but has a number of limitations," said Jose Carlos Gutiérrez-Ramos, the head of biotherapeutics R&D at Pfizer. "We are pleased to partner with Gliknik on this novel therapeutic approach which may provide an important new option in managing debilitating autoimmune diseases."

Sangamo's 'Zinc Finger' Bags Potential $320M Biogen Idec Blood Drug Deal

Jan 9, 2014

Shares of Sangamo BioSciences Inc. soared more than 30 percent Thursday as the company inked a potential $320 million deal with Biogen Idec Inc. to develop drugs for blood disorders such as sickle cell disease.

Sangamo will receive $20 million upfront to use its gene-editing zinc finger nuclease platform to fix the abnormal structure or underproduction of hemoglobin.

Cambridge, Mass.-based Biogen, led by former Exelixis Inc. CEO George Scangos, could pay out another $300 million to Sangamo in milestones.

Sangamo stock by mid-afternoon Thursday was up $4.82, or 35 percent, to $18.47 per share.

Sangamo will be responsible for early research and development and would have co-promotion rights in the United States for drugs coming out of the collaboration focusing on sickle cell disease and beta-thalassemia, a disorder that reduces the ability of blood cells to transport oxygen.

Sangamo last year won a $6.4 million grant from the California Institute for Regenerative Medicine to create a beta-thalassemia therapy.

"We are delighted to partner our hemoglobinopathies programs with Biogen Idec," Sangamo President and CEO Edward Lanphier said in a press release. "Biogen Idec is a leader in drug development and has a history of successfully translating cutting-edge science into treatments that provide life-changing clinical benefit for patients."

AbbVie continues deal spree with $405M CF tie-up with Galapagos

September 24, 2013

A day after AbbVie unveiled an $840 million tie-up to develop a new anti-inflammatory therapy, the pharma company is continuing its deal spree today with a $405 million discovery pact on cystic fibrosis with Belgium's Galapagos.

AbbVie wants to whip up some new programs that target the main mutations present in the cystic fibrosis population, with an eye to getting into the clinic next year. Galapagos gets a $45 million upfront with $360 million in a range of milestones.

Galapagos has been working with the Cystic Fibrosis Foundation since 2005 and tapped CF as its first orphan disease focus in 2010. The European biotech has also been working with AbbVie, spotlighting a JAK1 inhibitor for rheumatoid arthritis and expanding that deal to include Crohn's.

For AbbVie it's another opportunity to expand its pipeline in an effort to find a way to diversify its marketing effort. AbbVie relies on the megablockbuster Humira for most of its revenue, which makes analysts uneasy every time they inspect the numbers. For Galapagos the deal marks a further expansion of its already ambitious partnering effort. Galapagos has deals in place with GlaxoSmithKline, J&J and Servier in addition to AbbVie.

"We're pleased to enhance our partnership with Galapagos to include research in cystic fibrosis, a debilitating disease with significant unmet medical need. Our knowledge of the patient experience, combined with innovative advances in the understanding of disease etiology, offers the potential for new transformational treatments," said Jim Sullivan, vice president, pharmaceutical discovery, AbbVie.

Demonstrating PoR Will Inflect Significant Value Prior to Clinical PoC or Prior to Any Clinical Development

- Well supported evidence of biological relevance, e.g. target engagement, relevance of disease/indication (can also constitute pre-clinical PoC)

- Well supported, compelling TPP

- Well supported argument for commercial value of TPP
  - Includes argument for clear differentiation to support value; (especially critical if current or anticipated generic SoC exists)
    - Specific target patient segment with unmet need
    - Biomarker gets one to front of queue
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