Investor presentation
March 2018
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Ablynx highlights

- **Powerful Nanobody technology platform** combining the key advantages of mAbs and small molecules with more than 45 wholly-owned and partnered programmes (8 in clinical development and potentially 3 more in 2018)

- **Caplacizumab (anti-vWF), wholly-owned:** Compelling Phase III data in the ~€1.2Bn p.a. orphan aTTP market potentially supporting regulatory approval in the near-term in Europe and the USA

- **ALX-0171 (anti-RSV), wholly-owned:** Phase IIb trial in progress which could result in a potential breakthrough in the €1Bn+ RSV market, pioneering pulmonary delivery of biologics

- **Vobarilizumab (anti-IL-6R), partnership with AbbVie:** Encouraging results in Phase IIb RA studies with Phase IIb trial in SLE in progress

- **Multiple collaborations** with strategic partners, including Merck & Co., Inc. and Sanofi, which have the potential to generate >€10.6Bn in milestone payments plus royalties

- **Listed on Euronext Brussels and Nasdaq with liquidity¹ >€350M**

- **Sanofi made an offer on 29 January 2018 to acquire Ablynx for €3.9 billion – unanimously recommended by the Ablynx Board**

¹ cash, cash equivalents, restricted cash and other financial assets
Ablynx

The evolution of a biotech company

2001

Foundation

- €5M seed financing
- No products
- No partnerships
- 10 employees

2002

Technology platform

- €70M private equity
- €85M IPO Euronext
- 11 R&D projects
- 1 Nanobody in the clinic
- 3 partnerships

2007

R&D – early stage

- €70M private equity
- €85M IPO Euronext
- 11 R&D projects
- 1 Nanobody in the clinic
- 3 partnerships

2018

Prepping for commercialisation

- $230M US IPO completed in 2017
- Hybrid business model with 8 partnerships and >45 R&D programmes
- 8 Nanobodies in the clinic with >2,000 patients and volunteers treated
- First product (caplacizumab) planned to be launched this year
- >450 employees in Ghent, Belgium, with offices now in Philadelphia, USA
- Anticipating a future as part of the Sanofi family
## Broad product pipeline >45 programmes

### 8 Nanobodies in clinical development

<table>
<thead>
<tr>
<th>Product</th>
<th>Indication</th>
<th>Target</th>
<th>Pre-clinical</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
<th>Filing</th>
</tr>
</thead>
<tbody>
<tr>
<td>caplacizumab</td>
<td>aTTP</td>
<td>vWF</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>vobarilizumab</td>
<td>RA, SLE</td>
<td>IL-6R, IL-6R</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>ALX-0171</td>
<td>RSV</td>
<td>RSV</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Japan</td>
</tr>
</tbody>
</table>

1st programme: bispecific (Japan)
2nd programme: Immuno-Oncology (not disclosed)
Up to 15 programmes: various
Up to 8 programmes: Immuno-Inflammation (various)

<table>
<thead>
<tr>
<th>Product</th>
<th>Indication</th>
<th>Target</th>
<th>Pre-clinical</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
<th>Filing</th>
</tr>
</thead>
<tbody>
<tr>
<td>ozoralizumab</td>
<td>RA</td>
<td>TNFα</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Japan</td>
</tr>
<tr>
<td>ALX-0761/M1095</td>
<td>Psoriasis</td>
<td>IL-17A/IL-17F</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>ALX-1141/M6495</td>
<td>Osteoarthritis</td>
<td>ADAMTS-5</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>BI 836880</td>
<td>Oncology</td>
<td>VEGF/Ang2</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>BI 655088</td>
<td>Chronic kidney disease</td>
<td>CX3CR1</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>ALX-0141</td>
<td>Bone disorders</td>
<td>RANKL</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Greater China</td>
</tr>
</tbody>
</table>

>20 wholly-owned and partnered programmes

🌟 Filing in EU based on Phase II TITAN and Phase III HERCULES data
Ablynx

Diversified shareholder base – February 28, 2018

- Listed on Euronext Brussels and on Nasdaq (ABLX)
- 75.1M outstanding shares
- 2.7M outstanding warrants (in number of shares)

Estimated breakdown of share capital*

*based on public filings
Nanobodies

Derived from heavy-chain only antibodies

- *Camelid* heavy-chain only antibodies are stable and fully functional
- Nanobodies represent the next generation of antibody-derived biologics

**Ablynx’s Nanobody**
- small and robust
- easily linked together
- sequence homology comparable to humanised/human mAbs
- nano- to picomolar affinities
- able to bind and block challenging targets
- multiple administration routes
- manufactured in microbial cells

Conventional antibodies

Heavy chain only antibodies
Ablynx’s Nanobodies

Platform advantages

Mix and match
Multi-specific/multivalent Nanobodies that address multiple targets in a single drug molecule – flexible GS linker lengths

Multiple delivery routes
- Injection
- Inhalation
- Ocular
- Oral-to-topical

Manufacturing
High-yield, high-concentration, low-viscosity, microbial production

Able to bind and block challenging targets
Nanobodies against ion channels and GPCRs

Customised half-life extension
- Albumin-binding Nanobody
- Fc

Flexible GS linker lengths

Injection
Inhalation
Ocular
Oral-to-topical
Four lead programmes focussed on unmet clinical needs

Internal clinical development in c. 700 patients

**Caplacizumab (anti-vWF) in aTTP**
- Life-threatening blood clotting disease
- Up to 20% mortality & significant morbidity
- No therapeutic drug indicated
- Compelling Phase III HERCULES data
- Potential first launch in 2018
- €1.2Bn estimated market opportunity

**Vobarilizumab (anti-IL-6R) in SLE**
- Multi-organ inflammatory disease
- Many patients not adequately treated
- Significant morbidity & mortality
- Good rationale for targeting IL-6 pathway
- $2.2Bn market size predicted by 2025

**MAA filed in Europe – BLA filing in H1 2018**

**ALX-0171 (anti-RSV, inhaled) in infected hospitalised infants**
- Most common cause of viral respiratory infection in infants
- >3 million hospitalisations globally p.a.
- No therapeutic widely in use
- >€1Bn market opportunity

**ALX-0171 (anti-RSV, inhaled) in infected HSC transplant patients**
- RSV infection is a significant cause of morbidity & mortality
- ~50,000 allogeneic HSC transplants globally p.a.
- Infection rates of ~12%
- 20-30% mortality in a sub-segment of patients

**Phase IIb results in H2 2018**

**Phase II initiation in H1 2018**

1 Decision Resources 2016
### Broad range of partnerships

**Potential to generate >€10.6Bn in milestones plus royalties**

<table>
<thead>
<tr>
<th>Partner</th>
<th>Collaboration/Programmes</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>SANOFI</strong></td>
<td>• Strategic discovery</td>
<td>• Strategic discovery collaboration (up to 8 programmes) with focus on multi-s specifics in immune-mediated inflammatory diseases</td>
</tr>
<tr>
<td></td>
<td>collaboration (up to 8</td>
<td>• Strategic immuno-oncology collaboration (up to 17 programmes) with focus on multi-s specifics; first to enter clinical studies in Q2 2018</td>
</tr>
<tr>
<td></td>
<td>programmes)</td>
<td>• Ion channel collaboration (up to 2 programmes)</td>
</tr>
<tr>
<td><strong>MERCK</strong></td>
<td>• Global licensing option</td>
<td>• Global licensing option deal for vobarilizumab (anti-IL-6R) in RA and SLE</td>
</tr>
<tr>
<td></td>
<td>deal for vobarilizumab</td>
<td>• Opt-in decision expected in late Q2 2018</td>
</tr>
<tr>
<td></td>
<td>(anti-IL-6R) in RA and</td>
<td></td>
</tr>
<tr>
<td></td>
<td>SLE</td>
<td></td>
</tr>
<tr>
<td><strong>ABBVIE</strong></td>
<td>• Five active programmes</td>
<td>• Five active programmes of which one is moving towards Phase II and another is in Phase I</td>
</tr>
<tr>
<td></td>
<td>of which one is moving</td>
<td></td>
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<tr>
<td></td>
<td>towards Phase II and</td>
<td></td>
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<tr>
<td></td>
<td>another is in Phase I</td>
<td></td>
</tr>
<tr>
<td><strong>MERCK</strong></td>
<td>• Global strategic</td>
<td>• Global strategic alliance with four active programmes of which two are in Phase I</td>
</tr>
<tr>
<td></td>
<td>alliance with four</td>
<td></td>
</tr>
<tr>
<td></td>
<td>active programmes of</td>
<td></td>
</tr>
<tr>
<td></td>
<td>which two are in Phase I</td>
<td></td>
</tr>
<tr>
<td><strong>Boehringer Ingelheim</strong></td>
<td>• Discovery collaboration</td>
<td>• Discovery collaboration focussed on one multi-specific Nanobody programme</td>
</tr>
<tr>
<td></td>
<td>focussed on one multi-</td>
<td></td>
</tr>
<tr>
<td></td>
<td>specific Nanobody</td>
<td></td>
</tr>
<tr>
<td></td>
<td>programme</td>
<td></td>
</tr>
<tr>
<td><strong>NOVO NORDISK</strong></td>
<td>• Licensing deals in</td>
<td>• Licensing deals in Greater China for ALX-0141 (anti-RANKL) and ozoralizumab (anti-TNFα)</td>
</tr>
<tr>
<td></td>
<td>Greater China for</td>
<td></td>
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<tr>
<td></td>
<td>ALX-0141 (anti-RANKL) and</td>
<td></td>
</tr>
<tr>
<td></td>
<td>ozoralizumab (anti-TNFα)</td>
<td></td>
</tr>
<tr>
<td><strong>TAE SHO</strong></td>
<td>• Licensing deal in Japan</td>
<td>• Licensing deal in Japan for ozoralizumab in RA</td>
</tr>
<tr>
<td></td>
<td>for ozoralizumab in RA</td>
<td></td>
</tr>
</tbody>
</table>

>30 active partnered programmes; >€450M in non-dilutive cash received
Caplacizumab (anti-vWF) – wholly-owned

Potential first-in-class treatment for acquired thrombotic thrombocytopenic purpura (aTTP)
Caplacizumab – anti-vWF Nanobody
First-in-class potential for the treatment of aTTP

• The Opportunity:
  - Estimated annual market potential ~ €1.2Bn
  - Orphan Drug Status (EU/USA)
  - Patent protection potentially up to 2035 in certain jurisdictions

• Achievements 2017:
  - Marketing Authorization Application filed in Europe
  - Fast Track designation received from the FDA
  - Compelling results from Phase III HERCULES study
  - ASH late-breaking presentation and “Best of ASH”
  - Positive ethno-bridging data in Japanese healthy volunteers

• The Future:
  - 2018: anticipated BLA submission in the USA and first launch in Europe
  - 2019: anticipated launch in USA

aTTP: acquired thrombotic thrombocytopenic purpura
Caplacizumab (anti-vWF Nanobody) in aTTP

- aTTP is an acute, life-threatening autoimmune blood clotting disorder
- High unmet medical need with no approved therapeutic drug

Caplacizumab’s unique mode of action blocks binding of vWF to platelets which has an immediate effect on platelet aggregation and the ensuing micro-clot formation
Acquired TTP

Acute, life-threatening blood clotting disorder

**episode**

aTTP patient

Sudden onset in otherwise healthy person (nausea, fever, coma ...)

**diagnosis**

Emergency Room

Initial diagnosis based on thrombocytopenia & haemolysis

**treatment**

ICU/haematology unit

Plasma exchange until normalisation of platelet count + immune suppressants

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**the market**

- Ablynx estimates a total of 7,500 episodes of aTTP p.a. in North America, Europe and Japan
- No therapeutic drug approved for this indication
- Concentrated patient distribution

**acute and life-threatening**

- Extensive micro clot formation in small blood vessels
- Tissue ischemia, organ dysfunction, major thromboembolic events
- Up to 20% mortality rate in acute phase\(^1\) and up to 80% of patients suffer from recurrences\(^2\)

**important clinical needs**

- Faster resolution of acute episode of aTTP and related organ damage
- Reduce risk of mortality and thromboembolic events
- Prevent recurrences while on treatment
- Reduce risk of refractoriness to treatment
- Reduce dependency on PEX

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\(^{1}\) Allford *et al.*, BJH 2003, Kremer Hovinga, Blood 2010; Benhamou, Haematologica 2012; \(^{2}\) Thejeel *et al.* 2016; Falter *et al.* 2013
Phase III HERCULES study design
Randomised, double-blind, placebo-controlled, multi-national study

1 iv bolus (10mg) followed by daily sc (10mg); 2 including corticosteroids at start of daily PEX until underlying disease activity resolved; 3 patients who experience a recurrence restart daily PEX and are switched to open label caplacizumab

Primary endpoint: time to confirmed normalisation of platelet count response

Secondary endpoints:
- aTTP-related death, recurrence of aTTP, or at least one major thromboembolic event during the study drug treatment
- recurrence of aTTP in the overall study period
- refractoriness to treatment
- time to normalisation of three organ damage markers

PEX: plasma exchange
# Phase III HERCULES study

## Patient demographics and baseline disease characteristics

<table>
<thead>
<tr>
<th></th>
<th>Placebo N=73</th>
<th>Caplacizumab N=72</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Mean age (SD)</strong></td>
<td>47.3 (14.1%)</td>
<td>44.9 (13.5%)</td>
</tr>
<tr>
<td><strong>Females – N (%)</strong></td>
<td>51 (69.9%)</td>
<td>49 (68.1%)</td>
</tr>
<tr>
<td><strong>Baseline platelet count (10^9/L) – mean (SD)</strong></td>
<td>39.1 (29.1%)</td>
<td>32.0 (27.2%)</td>
</tr>
<tr>
<td><strong>Previous aTTP episode(s) – N (%)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>- initial</td>
<td>34 (46.6%)</td>
<td>48 (66.7%)</td>
</tr>
<tr>
<td>- recurrent</td>
<td>39 (53.4%)</td>
<td>24 (33.3%)</td>
</tr>
<tr>
<td><strong>ADAMTS13 activity at baseline – N (%)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>- &lt;10%</td>
<td>65 (90.3%)</td>
<td>58 (81.7%)</td>
</tr>
<tr>
<td>- ≥10%</td>
<td>7 (9.7%)</td>
<td>13 (18.3%)</td>
</tr>
<tr>
<td><strong>Disease severity at baseline – N (%)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>- less severe</td>
<td>48 (65.8%)</td>
<td>42 (58.3%)</td>
</tr>
<tr>
<td>- very severe</td>
<td>7 (34.2%)</td>
<td>30 (41.7%)</td>
</tr>
</tbody>
</table>

*very severe was defined as: French severity score ≥3 (cerebral involvement: yes=1 / no=0, LDH: >10xULN=1 / ≤10xULN=0, age: >60 years=2 / >40 and ≤60 years=1 / ≤40 years=0), or severe neurological involvement at baseline, or cardiac involvement (cTnI > 2.5 x upper limit of normal)*
Primary endpoint

Statistically significant reduction in time to platelet count response*

* platelet count response was defined as initial platelet count ≥ 150×10⁹/L with subsequent stop of daily PEX within 5 days
First key secondary endpoint

Subjects with aTTP-related death, aTTP recurrence or at least one major thromboembolic event during the study drug treatment period

<table>
<thead>
<tr>
<th>Number of subjects (%)</th>
<th>Placebo N=73</th>
<th>Caplacizumab N=72*</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Total number of subjects with at least one of the events below</strong>¹</td>
<td>36 (49.3%)</td>
<td>9 (12.7%)</td>
</tr>
<tr>
<td>aTTP-related death²</td>
<td>3 (4.1%)</td>
<td>0</td>
</tr>
<tr>
<td>recurrence³ of aTTP</td>
<td>28 (38.4%)</td>
<td>3 (4.2%)</td>
</tr>
<tr>
<td>at least one treatment emergent major thromboembolic event²:</td>
<td>6 (8.2%)⁴</td>
<td>6 (8.5%)</td>
</tr>
<tr>
<td><strong>p-value</strong></td>
<td></td>
<td>&lt;0.0001</td>
</tr>
</tbody>
</table>

* percentages are based on 71 subjects entering the study drug treatment period

¹ patients could have more than 1 event

² adjudication of aTTP-related death and major thromboembolic events by a blinded independent committee

³ recurrence = recurrent thrombocytopenia after initial recovery of platelet count, requiring re-initiation of daily PEX

⁴ 26 of 73 placebo treated patients had an exacerbation and were switched to open-label caplacizumab, potentially preventing additional treatment emergent major thromboembolic events during the study drug treatment period
## Second key secondary endpoint

### Subjects with aTTP recurrence during the overall study period

<table>
<thead>
<tr>
<th>Number of subjects (%)</th>
<th>Placebo N=73</th>
<th>Caplacizumab N=72*</th>
</tr>
</thead>
<tbody>
<tr>
<td>aTTP recurrence(^1)</td>
<td>28 (38.4%)</td>
<td>9 (12.7%)</td>
</tr>
<tr>
<td>during the study drug treatment period</td>
<td>28 (38.4%)</td>
<td>3 (4.2%)</td>
</tr>
<tr>
<td>during the follow-up period</td>
<td>0</td>
<td>6 (9.1%)(^2)</td>
</tr>
</tbody>
</table>

\(p\)-value: \(<0.001\)

*percentages are based on 71 subjects entering the study drug treatment period and 66 subjects in the follow-up period

\(^1\) recurrence = recurrent thrombocytopenia after initial recovery of platelet count, requiring re-initiation of daily PEX

\(^2\) ADAMTS-13 activity levels were <10% at the end of the study drug treatment period in all of these patients
### Third key secondary endpoint

#### Percentage of subjects with refractory aTTP

<table>
<thead>
<tr>
<th></th>
<th>Placebo N=73*</th>
<th>Caplacizumab N=72</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>RefRACTORY aTTP</strong>¹</td>
<td>3 (4.2%)</td>
<td>0</td>
</tr>
<tr>
<td><strong>p-value</strong></td>
<td>0.057</td>
<td></td>
</tr>
</tbody>
</table>

¹ refractory TTP = absence of platelet count doubling after 4 days of standard treatment and LDH > ULN

<table>
<thead>
<tr>
<th></th>
<th>Placebo N=73*</th>
<th>Caplacizumab N=72</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>RefRACTORY aTTP</strong>²</td>
<td>5 (7.0%)</td>
<td>0</td>
</tr>
<tr>
<td><strong>p-value</strong></td>
<td>0.018</td>
<td></td>
</tr>
</tbody>
</table>

² refractory TTP = persistent thrombocytopenia, lack of a sustained platelet count increment or platelet counts of <50 x 10⁹/L and a persistently raised LDH level (> 1.5 ULN) despite five plasma exchanges and steroid treatment

* one subject discontinued prior to day 5 and is not included in the analysis

**Protocol-specified key secondary endpoint** *(Benhamou et al., 2015)*

**International TTP working group consensus definition** *(Scully et al., 2017)*

**LDH:** lactate dehydrogenase; **ULN:** upper limit of normal
Fourth key secondary endpoint

Time to normalisation of organ damage markers

% of subjects with organ damage markers >ULN at baseline

<table>
<thead>
<tr>
<th>Marker</th>
<th>% of subjects with organ damage markers</th>
</tr>
</thead>
<tbody>
<tr>
<td>lactate dehydrogenase</td>
<td>87.1%</td>
</tr>
<tr>
<td>cardiac Troponin I</td>
<td>53.8%</td>
</tr>
<tr>
<td>serum creatinine</td>
<td>22.7%</td>
</tr>
</tbody>
</table>

all subjects N=145

\[1\] time to LDH ≤ 1 x ULN and cardiac Troponin I ≤ 1 x ULN and serum creatinine ≤ 1 x ULN
## Other secondary endpoints

### Plasma exchange parameters, duration of ICU stay and overall hospitalisation

<table>
<thead>
<tr>
<th>Overall study drug treatment period (mean±SE)</th>
<th>Placebo N=73¹</th>
<th>Caplacizumab N=71</th>
<th>% relative reduction</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of days of plasma exchange</td>
<td>9.4±0.8</td>
<td>5.8±0.5</td>
<td>↓38%</td>
</tr>
<tr>
<td>Volume of plasma (L)</td>
<td>35.9±4.2</td>
<td>21.3±1.6</td>
<td>↓41%</td>
</tr>
<tr>
<td>Number of days in intensive care unit</td>
<td>9.7±2.1</td>
<td>3.4±0.4</td>
<td>↓65%</td>
</tr>
<tr>
<td></td>
<td>(n=27)</td>
<td>(n=28)</td>
<td></td>
</tr>
<tr>
<td>Number of days in hospital</td>
<td>14.4±1.2</td>
<td>9.9±0.7</td>
<td>↓31%</td>
</tr>
</tbody>
</table>

¹ 26 of 73 placebo treated patients had an exacerbation and were switched to open-label caplacizumab, potentially reducing the mean values on plasma exchange parameters and duration of ICU stay and overall hospitalisation during the study drug treatment period.
## Safety profile

### Treatment emergent adverse events (TEAEs)

<table>
<thead>
<tr>
<th>Number of subjects (%) with TEAE</th>
<th>Placebo N=73</th>
<th>Caplacizumab N=71</th>
</tr>
</thead>
<tbody>
<tr>
<td>At least one TEAE</td>
<td>71 (97.3%)</td>
<td>69 (97.2%)</td>
</tr>
<tr>
<td>At least one study drug-related TEAE</td>
<td>32 (43.8%)</td>
<td>41 (57.7%)</td>
</tr>
<tr>
<td>At least one TEAE leading to study drug discontinuation</td>
<td>9 (12.3%)</td>
<td>5 (7.0%)</td>
</tr>
<tr>
<td>At least one SAE</td>
<td>39 (53.4%)</td>
<td>28 (39.4%)</td>
</tr>
<tr>
<td>At least one study drug-related SAE</td>
<td>4 (5.5%)</td>
<td>10 (14.1%)</td>
</tr>
<tr>
<td>At least one SAE leading to death</td>
<td>3 (4.1%)</td>
<td>1 (1.4%)¹</td>
</tr>
</tbody>
</table>

¹ adverse event occurred during the follow-up period of the study and was assessed by the investigator as not related to study drug treatment

Safety data consistent with Phase II TITAN study
# Safety profile

## Bleeding-related TEAEs*

<table>
<thead>
<tr>
<th>Number of subjects (%) with TEAE</th>
<th>Placebo N (%)</th>
<th>Caplacizumab N (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>**Bleeding-related TEAEs (by SMQ)**¹</td>
<td>17 (23.3%)</td>
<td>33 (45.6%)</td>
</tr>
<tr>
<td>Epistaxis</td>
<td>1 (1.4%)</td>
<td>17 (23.9%)</td>
</tr>
<tr>
<td>Gingival bleeding</td>
<td>0</td>
<td>8 (11.3%)</td>
</tr>
<tr>
<td>Bruising</td>
<td>3 (4.1%)</td>
<td>5 (7.0%)</td>
</tr>
<tr>
<td>Hematuria</td>
<td>1 (1.4%)</td>
<td>4 (5.6%)</td>
</tr>
<tr>
<td>Vaginal hemorrhage</td>
<td>1 (1.4%)</td>
<td>3 (4.2%)</td>
</tr>
<tr>
<td>Menorrhagia</td>
<td>1 (1.4%)</td>
<td>2 (2.8%)</td>
</tr>
<tr>
<td>Catheter site hemorrhage</td>
<td>3 (4.1%)</td>
<td>2 (2.8%)</td>
</tr>
<tr>
<td>Injection site bruising</td>
<td>2 (2.7%)</td>
<td>2 (2.8%)</td>
</tr>
<tr>
<td>Hematochezia</td>
<td>0</td>
<td>2 (2.8%)</td>
</tr>
<tr>
<td>Hematoma</td>
<td>0</td>
<td>2 (2.8%)</td>
</tr>
</tbody>
</table>

* Treatment emergent adverse events occurring in at least 2 subjects in either group

¹ Standardised MeDRA Query “Hemorrhage”
Conclusions

- Faster resolution of an aTTP episode with significantly shorter time to platelet count response
- Clinically relevant reduction in aTTP-related death, exacerbation of aTTP, or a major thromboembolic event
- Prevention of aTTP relapses when treatment is extended until resolution of underlying disease
- Potential to prevent refractory disease and speed normalisation of markers of organ damage
- Striking reduction in use of plasma exchange and length of stay in the ICU and hospital, potentially providing considerable cost savings
- Safety profile in line with previous study results and mechanism of action

Caplacizumab addresses the pathophysiological platelet aggregation that leads to the formation of microthrombi and the resultant mortality and morbidity seen in aTTP
Next steps

All communicated timelines remain on track

• Complete full analysis of Phase III HERCULES results and present at a key scientific conference ✓ and submit to a peer-reviewed journal

• Submit Phase III HERCULES results to EMA to support the MAA filed in February 2017 ✓

• Prepare BLA for USA FDA, filing planned in H1 2018

• Continue the 3-year follow-up study for eligible patients who participated in the Phase III HERCULES study

• Continue preparations for commercialisation of caplacizumab
Preparing the market for caplacizumab
Planned launch in Europe in 2018

<table>
<thead>
<tr>
<th>Medical Affairs</th>
<th>Pricing &amp; Access</th>
<th>Commercial Operations</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Medical Science Liaisons (MSLs)</td>
<td>• Payor landscape</td>
<td>• Key Account Managers (KAMs)</td>
</tr>
<tr>
<td>• Key centres &amp; stakeholders</td>
<td>• Value proposition</td>
<td>• Contract Sales Organisation</td>
</tr>
<tr>
<td>• Advisory boards</td>
<td>• Health economic model</td>
<td>• U.S.A. office</td>
</tr>
<tr>
<td>• Diagnostic &amp; Treatment guidelines</td>
<td>• Early access programmes</td>
<td>• Stock in reference centres</td>
</tr>
<tr>
<td>• Registries</td>
<td>• First launch expected in Germany</td>
<td>• Next day delivery (24/7)</td>
</tr>
<tr>
<td>• Patient advocacy</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
# Caplacizumab in aTTP

## Key anticipated milestones

<table>
<thead>
<tr>
<th>Year</th>
<th>2017</th>
<th>2018</th>
<th>2019</th>
<th>2020</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Clinical trials</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Japanese Phase I results</td>
<td></td>
<td></td>
<td>3-year follow-up study results</td>
</tr>
<tr>
<td></td>
<td>Phase III HERCULES topline results</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Regulatory EMA</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>MAA submission</td>
<td>MA</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Regulatory US FDA</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td>BLA submission</td>
<td>BLA approval</td>
<td></td>
</tr>
<tr>
<td><strong>Operations</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>First sales EU</td>
<td>First sales USA</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>MSL</td>
<td>KAM</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Abbreviations:**
- **MAA**: marketing authorisation application
- **MA**: marketing authorisation
- **BLA**: biologic license application
- **MSL**: medical science liaison
- **KAM**: key account manager
Inhaled ALX-0171 (anti-RSV) – wholly-owned

Potential first-in-class treatment for RSV infections
ALX-0171 – inhaled anti-RSV Nanobody

Potential breakthrough for the treatment of RSV infections

- **RSV opportunity:**
  - >€1Bn market opportunity
  - No widely used therapeutic available
  - Patent protection up to 2037 in certain jurisdictions

- **Infants – Phase IIb RESPIRE study:**
  - RSV-infected hospitalised infants
  - first 3 safety cohorts complete (36 infants) – positive DMC recommendation
  - parallel dose part initiated (144 infants)
  - results anticipated in H2 2018

- **Adults – Phase II study:**
  - RSV-infected hematopoietic stem cell transplant patients
  - Planned clinical trial start in H1 2018

RSV: respiratory syncytial virus; DMC: Data Monitoring Committee
RSV infections – vulnerable populations

Unmet medical need and no approved therapeutic

INFANTS
out-patient and hospitalised settings

- 33.1 million episodes globally in 2015
- 3.2 million hospital admissions globally in 2015
- 48,000-74,500 in-hospital deaths globally in children <5y in 2015
- up to 118,200 overall RSV-related deaths globally in 2015
- long-term disease burden

ELDERLY
out-patient and hospitalised settings

- serious health risk for elderly
- 5.5% average infection rate p.a. with up to 16% hospitalised and 4-8% deaths
- USA: >170,000 hospital admissions and approximately 14,000 deaths p.a.
- high disease burden in nursing homes

STEM CELL TRANSPLANT PATIENTS
hospitalised setting

- approximately 50,000 allogeneic haematopoietic stem cell transplant (HSCT) procedures globally p.a.
- about 12% become infected with RSV within 1 year
- approximately 40% of those infected with RSV progress to pneumonia and lower respiratory tract infection (LRTI) with an associated mortality rate of 20-30%

# Anti-RSV Nanobody – ALX-0171

## Incorporating unique Nanobody platform advantages

<table>
<thead>
<tr>
<th>Platform advantage</th>
<th>Product features</th>
</tr>
</thead>
<tbody>
<tr>
<td>Multivalent formatting</td>
<td>• 3 Nanobodies linked together that bind to the F-protein of RSV</td>
</tr>
<tr>
<td></td>
<td>• &gt;6,000 fold increase in potency over monovalent construct</td>
</tr>
<tr>
<td></td>
<td>• Up to 10,000 fold reduction in viral titres <em>in vitro</em></td>
</tr>
<tr>
<td></td>
<td>• Neutralises 87% of a broad range of clinical RSV isolates</td>
</tr>
</tbody>
</table>

| Delivery via inhalation     | • Biological activity maintained after nebulisation                              |
|                             | • Delivered directly to the site of infection                                     |
|                             | • Very encouraging efficacy in neonatal lamb model for infant RSV infection       |
|                             | • Results of Phase I studies in adults supported further development             |
|                             | • Well-tolerated in hospitalised, RSV-infected infants                           |
Inhaled ALX-0171

Promising results in neonatal lamb model for infant RSV*

** Composite assessment of disease related parameters such as weakness, depression, lethargy, drooping of ears and not eating

- RSV Symposium, November 2014
- Strong therapeutic effect and markedly reduced gross lung viral lesions demonstrated following 3 consecutive daily administrations
Inhaled ALX-0171

Successfully completed Phase I/IIa study in 53 infants with a RSV infection

- Recruitment from Q4 2014 to Q2 2016
- Study centres in Europe and Asia-Pacific region
- Adapted infant inhalation device (vibrating mesh)
- Inhaled ALX-0171 administered once/day, for 3 consecutive days

Primary endpoint:
Safety and tolerability of ALX-0171

Secondary endpoints:
Assessment of clinical effect (feeding, respiratory rate, O₂ saturation, wheezing, coughing, general appearance), PD, PK and immunogenicity
First-in-infant Phase I/IIa study

Safety and tolerability

<table>
<thead>
<tr>
<th></th>
<th>Open-label group ALX-0171 (N=5)</th>
<th>Randomised group ALX-0171 (N=30)</th>
<th>Randomised group Placebo (N=16)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Adverse events (AEs)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>- number (%) of subjects with an AE</td>
<td>4 (80.0)</td>
<td>9 (30.0)</td>
<td>4 (25.0)</td>
</tr>
<tr>
<td>- number (%) of subjects with a treatment-related AE</td>
<td>1 (20.0)</td>
<td>2 (6.7)</td>
<td>0 (0.0)</td>
</tr>
<tr>
<td><strong>Serious adverse events (SAEs)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>- number (%) of subjects with an SAE</td>
<td>3* (60.0)</td>
<td>1** (3.3)</td>
<td>0 (0.0)</td>
</tr>
<tr>
<td>- number (%) of subjects with treatment-related SAEs</td>
<td>0 (0.0)</td>
<td>0 (0.0)</td>
<td>0 (0.0)</td>
</tr>
</tbody>
</table>

* 1 of whom discontinued  
** subject discontinued

- Most common AEs were infections and respiratory disorders
- 3 AEs related to ALX-0171: mild cough, mild rhinorrhoea, mild fever 11 days after last dose
- 5 SAEs reported: hypo-responsiveness, hypotonia, pneumonia (2) and atelectasis

Favourable safety and tolerability profile confirmed in the target population
First-in-infant Phase I/IIa study

Key secondary objectives

- Treatment with inhaled ALX-0171 had an immediate and significant impact on viral replication.
- Encouraging initial indication of therapeutic effect was demonstrated.

"median time to undetectable virus was >24 hours quicker with ALX-0171"
nominal p-value=0.014

"difference in effect on clinical score is statistically significant"
nominal p-value=0.0092

* Overall disease severity assessment including feeding intolerance, medical intervention, respiratory difficulty, respiratory frequency, apnoea, general condition and fever.
Inhaled ALX-0171

Phase IIb RESPIRE clinical efficacy study in 180 hospitalised infants

- Study start: Jan 2017; expected results: H2 2018
- Adapted infant inhalation device (vibrating mesh)
- Infants: 28 days to <2 years of age
- Inhaled ALX-0171 (3 doses) administered once/day, for 3 consecutive days

**Primary endpoint:** Anti-viral effect

**Secondary endpoints:** Assessment of clinical effect over time (feeding, respiratory rate, O₂ saturation, wheezing, coughing, general appearance), time to clinical response, effect on composite clinical scores, PD, PK and immunogenicity; safety

- 3:1
  - ALX-0171 (3 mg/kg) N=9
  - Placebo N=3
  - DMC safety review ✓
- 1:1:1:1
  - ALX-0171 (3 mg/kg) N=36
  - ALX-0171 (6 mg/kg) N=36
  - ALX-0171 (9 mg/kg) N=36
  - Placebo N=36
  - DMC safety review ✓
ALX-0171 development plan

Key potential near term milestones

<table>
<thead>
<tr>
<th>Year</th>
<th>Event Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>2017</td>
<td>Phase IIb – ongoing</td>
</tr>
<tr>
<td></td>
<td>Infants hospitalised with RSV (N=180)</td>
</tr>
<tr>
<td>2018</td>
<td>First patient dosed</td>
</tr>
<tr>
<td></td>
<td>Topline results</td>
</tr>
<tr>
<td>2019</td>
<td>IND / CTA submission</td>
</tr>
<tr>
<td></td>
<td>Topline results</td>
</tr>
<tr>
<td>2020</td>
<td>Potential co-development with partner</td>
</tr>
</tbody>
</table>

**Phase II in Japan**
Hospitalised infants with RSV (N=60)

**Phase II**
RSV-infected, hospitalised haematopoietic stem cell transplant patients (N=75)
Vobarilizumab (anti-IL-6R) – collaboration with AbbVie
Potential novel treatment for RA and SLE
Vobarilizumab – anti-IL-6R Nanobody

Potential novel treatment for RA and SLE

- Opportunity in multi-billion dollar markets
- RA:
  - encouraging results from two Phase IIb RA studies (N=596)
  - open-label extension study ongoing
- SLE
  - chronic multi-system inflammatory disease with unmet medical need
  - Phase II study ongoing (N=312); topline results in H1 2018
- Awaiting the SLE data and AbbVie’s opt-in decision before deciding on our future strategy

RA: rheumatoid arthritis; SLE: systemic lupus erythematosus
Rheumatoid Arthritis (RA)

Positive Phase IIb study results with vobarilizumab reported

- As a combination therapy with MTX, vobarilizumab had a positive impact on clinical efficacy endpoints after 24 weeks of treatment
  - high ACR50 and ACR70 responses of up to 61% and 45%
  - up to 70% of the patients achieved low disease activity* based on DAS28_{CRP} and up to 51% were in remission**

- As a monotherapy, up to 50% more patients achieved DAS28_{CRP} remission with vobarilizumab compared to tocilizumab (41% vs. 27%) after 12 weeks of treatment

- Vobarilizumab showed a favorable safety profile and has the potential for monthly administration

Vobarilizumab has potential for disease modifying activity in the management of RA

*DAS28_{CRP} ≤ 3.2  **DAS28_{CRP} <2.6
Systemic Lupus Erythematosus (SLE)

Auto-antibodies are the hallmark of the disease

- Chronic multi-organ inflammatory disease with serious pathological conditions
  - joints and skin are mostly affected
  - patients suffer from severe cardiovascular, CNS and renal complications
  - fluctuating disease activity with flares
  - diverse and individualised therapeutic approach
  - prevalence 20-150 per 100,000\(^1\); continues to grow
  - predominantly affects women; mostly diagnosed between 16 and 55 years old
  - more common and more severe in African-Americans, Hispanics and Asian women

---

\(^1\) Lawrence et al., Arthritis Rheum., 1998; Chakravarty et al., Arthritis Rheum., 2007; Pons-Estel et al., Semin Arthritis Rheum., 2010
Vobarilizumab in SLE

Compelling opportunity

• SLE market estimated to grow to $2.2 billion by 2025\(^1\)

• Current therapies are not always effective and have many side-effects
  - corticosteroids, anti-malarials and immunosuppressants commonly used
  - Benlysta\(^\circledR\) (belimumab) approved since 2011 but often with restricted access
  - Rituxan\(^\circledR\)/MabThera\(^\circledR\) (rituximab) used off-label

• Unmet need
  - reduction of disease activity
  - steroid sparing to reduce risk of future organ damage
  - prevention of flares
  - reduction of cardiovascular mortality
  - improved health-related quality-of-life

\(^1\) Decision Resources 2016
Vobarilizumab in SLE

Rationale for approach

- IL-6 levels are elevated in patients with SLE
- Inhibition or knock-out of IL-6R in pre-clinical models had a strong impact on the SLE phenotype
- Clinical studies targeting both IL-6 and IL-6R have shown encouraging preliminary efficacy data in SLE, though safety appears to have presented problems
- We have been able to learn from previous studies in designing the STEADY trial, particularly with regard to enrollment criteria
- Vobarilizumab has shown in the RA studies that it targets IL-6R effectively and has a favourable safety profile

Ball et al., Lupus, 2013; Tackey et al., Lupus, 2004; Mao et al., Clin Rheumatol., 2014; Wallace et al., Ann Rheum Dis, 2017; Illei et al., Arthritis & Rheumatism; 2010
Vobarilizumab in SLE

Phase II STEADY study on track

- Subjects with moderate-to-severe active, seropositive SLE
- Worldwide, randomised, double-blind, placebo-controlled 48 week dose-range finding study
- Patient recruitment completed in December 2016; topline data expected in H1 2018

Randomisation: 1:1:1:1:1

- Placebo
- ALX-0061 dose 1, Q4W
- ALX-0061 dose 2, Q4W
- ALX-0061 dose 2, Q2W
- ALX-0061 dose 3, Q2W

Primary endpoint at Week 24: modified (m)BICLA* response

Secondary endpoints:
- (m)BICLA, (m)SRI, (m)SLEDAI-2K and BILAG over time; patient’s and physician’s global assessment; flare rate; corticosteroid reduction

Other assessments:
- pharmacokinetics, pharmacodynamics, safety/tolerability, immunogenicity

* complement score omitted due to mechanism of action of vobarilizumab
Major collaborations with Merck & Co, Inc. and Sanofi

Exploring novel treatment approaches with multi-specific Nanobodies
Current major collaborations
Built on multi-specific capability

Leader in the immuno-oncology (IO) field:
- ~80% of total R&D budget\(^1\) invested in IO
- Keytruda\(^{®}\):
  - approved in 2014
  - >160 clinical studies in >30 tumor types

Strategic IO collaboration:
- Started Feb ‘14; expanded in July ‘15
- Up to 17 fully-funded programmes, targeting multiple immune-checkpoint modulators
- €33M upfront and €6M in pre-clinical milestone payments received to date
- Up to €5.7Bn in potential future milestones plus royalties
- First two clinical studies expected to start in 2018

Focus on immuno-inflammatory diseases:
- Chronic disorders (e.g. asthma, COPD, RA, AD, psoriasis) associated with significant morbidity, mortality and reduced quality of life
- Growing need for novel treatments to modify disease state
- Affects 5–7% of western populations\(^2\)
- Market expected to grow to $74Bn in 2022

Strategic discovery collaboration:
- Deal signed in July 2017
- Up to 8 different targets or target combinations
- €23M in upfront and €13M in option exercise fees received, plus research funding for 5 selected target combinations
- Potential additional option exercise fees
- Up to €2.4Bn in potential future milestones plus royalties

---

\(^{1}\) Bryan Garnier Oct 2015; \(^{2}\) GBI research Dec 2015

COPD: chronic obstructive pulmonary disease; RA: rheumatoid arthritis; AD: atopic dermatitis
Conclusion
## Summary of key upcoming potential milestones

<table>
<thead>
<tr>
<th><strong>Caplacizumab</strong> (anti-vWF)</th>
<th><strong>ALX-0171</strong> (anti-RSV)</th>
<th><strong>Vobarilizumab</strong> (anti-IL-6R)</th>
<th><strong>Other programmes</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>• H1’18: Planned BLA filing in the USA</td>
<td>• H1’18: Initiation of clinical study in Japanese RSV infected infants expected</td>
<td>• H1’18: Phase II STEADY topline results in SLE expected</td>
<td>• 2018: expect multiple Phase I, II and III trials to be initiated in our partnered programmes</td>
</tr>
<tr>
<td>• H2’18: Possible Marketing Authorisation in Europe</td>
<td>• H1’18: Initiation of clinical study in RSV infected HSC transplant patients expected</td>
<td>• 2018: AbbVie opt-in decision on SLE and RA</td>
<td>• 2018: anticipate readouts for several partnered clinical stage assets</td>
</tr>
<tr>
<td>• H2’18: Potential first commercial sales of caplacizumab in Germany</td>
<td>• H2’18: Phase IIb RESPIRE topline results in RSV infected infants expected</td>
<td>• 2018: If AbbVie doesn’t opt-in, explore new partnership opportunities</td>
<td>• 2018: possible expansion of existing partnerships or addition of new partners</td>
</tr>
</tbody>
</table>

### Diverse Nanobody portfolio with multiple near-term value creating catalysts
Questions

CONTACT DETAILS
Investor Relations +32 9 262 00 00 investors@ablynx.com www.ablynx.com