ABLYNX REPORTS POSITIVE TOP LINE RESULTS FOR ITS INHALED ANTI-RSV NANOBODY (ALX-0171) IN A PHASE I/IIa STUDY IN INFANTS HOSPITALISED WITH AN RSV INFECTION

- Primary endpoint of safety and tolerability met
- Anti-viral effect demonstrated and encouraging initial indication of therapeutic effect
- Results support advancing the programme into a Phase II efficacy study in infants
- Potential general proof-of-principle for the administration of Nanobodies® by inhalation

![Conference call and webcast today at 4pm CET/10am EST](http://www.ablynx.com/news/events-presentations/)

GHENT, Belgium, 3 May 2016 - Ablynx [Euronext Brussels: ABLX; OTC: ABYLY] today announced positive top line results from the first-in-infant Phase I/IIa study of its wholly-owned, inhaled, anti-RSV Nanobody, ALX-0171, in 53 infants, aged 1-24 months, hospitalised as a result of a respiratory syncytial virus (RSV) infection.

The study met its primary endpoint, demonstrating the favourable safety and tolerability profile of ALX-0171 when administered daily by inhalation in the target infant population, with no treatment-related serious adverse events reported.

ALX-0171 was detected in the serum of subjects after treatment, consistent with lung exposure. Anti-drug antibodies had no effect on the pharmacokinetics and no relation with adverse events was seen. Treatment with inhaled ALX-0171 had an immediate impact on viral replication and also reduced viral load, as compared to placebo. Analysis of a composite of clinical efficacy endpoints, the Global Severity Score\(^1\), led to an encouraging initial indication of a therapeutic effect for infants treated with ALX-0171.

**Dr Robert K. Zeldin, CMO of Ablynx, commented:** “We believe we are pioneers in the development of a treatment for infants infected with RSV, an area of significant unmet medical need. We are very pleased that the positive safety and tolerability profile of our inhaled Nanobody observed in adults has now been confirmed in this vulnerable target population. In addition, we demonstrated ALX-0171’s marked anti-viral effect in RSV-infected infants and saw encouraging initial signs of clinical efficacy. We believe these results strongly support advancement into a Phase II efficacy study in infants.”

**Dr Steve Cunningham, Department of Respiratory and Sleep Medicine, Royal Hospital for Sick Children, Edinburgh, UK, and Principle Investigator of the study, added:** “RSV infection is the most common cause of lower respiratory tract disease leading to hospital admission in infants. It is often associated with continued coughing or wheezing after the acute phase of the infection and evidence is accumulating that it increases the risk of asthma later in life. There is no effective therapy available at present and the positive outcome from Ablynx’s first-in-infant study is therefore an important step forward in the development of an effective therapeutic for this serious infection.”

**Webcast and presentation**

Ablynx will host a conference call/webcast today at 4 pm CET, 10 am EST. The webcast may be accessed by clicking [here](http://www.ablynx.com/news/events-presentations/). To participate in the Q&A, please dial +32 (0)2 402 3092, using confirmation code 6340391. Shortly thereafter, a replay of the webcast will be available on the Company’s website: [http://www.ablynx.com/news/events-presentations/](http://www.ablynx.com/news/events-presentations/).

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\(^1\) Poster presentation, Justicia et al: “Development and validation of a new clinical scale for infants suffering from acute respiratory infection”
About the first-in-infant Phase I/IIa study
The first-in-infant Phase I/IIa study recruited 53 hospitalised RSV-infected infants, aged 1-24 months, in multiple clinical centres in Europe and the Asia-Pacific region. The study consisted of an open-label lead-in phase with 5 infants, aged 5-24 months who received ALX-0171 and a double-blind, placebo-controlled phase with 48 infants, aged 1-24 months, who were randomised to ALX-0171 or placebo.

The primary endpoint of the study was the assessment of safety and tolerability for once daily inhalation of ALX-0171 for three consecutive days. Secondary endpoints included assessments of clinical effect (including effect on feeding, oxygen saturation, respiratory rate, wheezing, coughing and general appearance), immunogenicity, pharmacokinetics (PK) and pharmacodynamics (PD) of inhaled ALX-0171.

Serum samples were collected for PK analysis on the last day of treatment (day 3) post-dose. Anti-viral activity (viral load and time to undetectable virus) was measured in nasal swabs (plaque assay and qRT-PCR\(^2\)), prior to dosing and then 6 hours post-dose on each day of treatment. An analysis of the clinical effect used the Global Severity Score, a clinical score that categorises infants with respiratory infections based on 7 parameters: feeding intolerance, medical intervention, respiratory difficulty, respiratory frequency, apnoea, general condition and fever. The data collected in the study were matched to corresponding parameters in the Global Severity Score to assess the time course of disease severity in the treated group versus placebo.

About RSV and ALX-0171
Respiratory syncytial virus (RSV) is the most common cause of lower respiratory tract infections and the leading viral cause of severe lower respiratory tract disease in infants and young children worldwide. It is the primary cause of infant hospitalisation and virus associated deaths in infants, with estimated global annual infection and hospitalisation rates of 34 million and 3-4 million respectively\(^3\). It is associated with an estimated 3,000-8,500 deaths in infants <2 years globally per year\(^4\). In addition, RSV infections have been linked to an increased risk of asthma development later in life\(^5\). Current treatment of RSV infections is primarily focussed on symptomatic relief, hence the need for an effective and specific anti-RSV therapeutic.

Ablynx’s ALX-0171 has been developed to address this unmet medical need and is a potential breakthrough for the treatment of RSV infection in infants. This wholly-owned trivalent Nanobody binds to the F-protein of RSV, thereby inhibiting viral replication and neutralising RSV activity by blocking virus uptake into cells. The physical robustness of the Nanobody allows administration via inhalation directly to the site of infection, i.e. the respiratory tract including the lungs. ALX-0171 has shown a potent anti-viral effect against a broad range of RSV strains \textit{in vitro} and it has demonstrated a marked therapeutic effect following administration via nebulisation in a neonatal animal model for infant RSV infection\(^6\). Repeated daily inhalation of ALX-0171 was proven to be well-tolerated in multiple Phase I clinical studies in adults, including a study in subjects with hyper-reactive airways.

About Ablynx
\textbf{Ablynx} is a biopharmaceutical company engaged in the development of \textit{Nanobodies\(^\text{\textregistered}\)}, proprietary therapeutic proteins based on single-domain antibody fragments, which combine the advantages of conventional antibody drugs with some of the features of small-molecule drugs. Ablynx is dedicated to creating new medicines which will make a real difference to society. Today, the Company has more than 40 proprietary and partnered programmes in development in various therapeutic areas including inflammation, haematology, immuno-oncology, oncology and respiratory disease. The Company has

\(^1\) Real-time reverse transcription polymerase chain reaction, a method to detect RNA expression
\(^2\) Nair et al, Lancet 2010
\(^3\) Byington et al, Pediatric 2014
\(^5\) Oral presentation at the 9\textsuperscript{th} International RSV Symposium (November 2014); presentation available on the Ablynx website
collaborations with multiple pharmaceutical companies including AbbVie, Boehringer Ingelheim, Eddingpharm, Genzyme, Merck & Co., Inc., Merck KGaA, Novartis, Novo Nordisk and Taisho Pharmaceuticals. The Company is headquartered in Ghent, Belgium. More information can be found on www.ablynx.com.

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