



## **Mechanism of action of ABIVAX's First-in-class anti-HIV drug published today in peer-reviewed journal *Retrovirology***

**Novel mechanism of action - prevention of viral RNA exit from nucleus of infected cells - associated to unique anti-viral properties**

**Unlike any current anti-HIV drug:**

- **Drop of viral load induced by ABX464 is sustained following treatment cessation**
- **No HIV mutants emerge from ABX464 treatment**

**On-going phase II clinical trial in patients**

**Paris, April 13th, 2015** - ABIVAX, a clinical phase II stage biotechnology company developing first-in-class anti-viral drugs and vaccines, announced publication today of the mechanism of action of ABX464 in the April issue of *Retrovirology*: <http://www.retrovirology.com/content/12/1/30/abstract>. ABX464 is a novel small molecule in phase II clinical trial that inhibits HIV replication through an entirely new mechanism.

The pioneering research described in the scientific publication was conducted by six premier research institutions from France, Canada and Switzerland, in addition to the ABIVAX laboratories, under the leadership of Professor Jamal Tazi of the Molecular Genetics Institute at CNRS in Montpellier, France. The research team was able to demonstrate that:

1. ABX464 blocks viral replication by preventing the export of viral RNA from the nucleus to the cytoplasm in infected cells. This transport is normally mediated by a viral protein called Rev, and the activity of Rev is efficiently inhibited by ABX464. Never targeted before, Rev has been postulated of potential interest for HIV treatment for some time, but ABX464 is the first molecule under development aimed at inhibiting it.

Jamal Tazi, senior author of the article, stresses that *"ABX464 targets an event after the genetic material of the virus has been integrated in the cell. This way, ABX464 not only prevents the infection of new cells, but it is the only drug to date that can act on already infected cells and prevent the synthesis of new viruses."* He added, *"Therefore the difficult-to-reach reservoir of infected cells remaining under current HIV therapy may become reachable."*

ABX464 leads to a significant and sustained reduction of viral load, which lasts for several weeks after cessation of treatment in a preclinical reference model. In contrast, the control group, receiving current state of the art triple anti-HIV therapy, had an immediate rebound of the viral load after stopping the treatment. Current treatments in patients merely hold the virus at bay whereas ABX464 may lead to durable suppression of viral load.

2. ABX464 does not affect the physiological cellular RNA-processing in humans. This is an important finding, as it suggests that ABX464 is specific for HIV RNAs and does not influence the synthesis of human proteins.
3. ABX464 does not lead to HIV mutants that become resistant to treatment. Thus, in contrast to all other anti-HIV drugs, ABX464 may be effective as a monotherapy.

Professor Mark Wainberg, former President of the International AIDS Society and co-author of the publication, said *"We are very encouraged by the data obtained with ABX464, which has the potential to become part of a functional cure strategy for patients with HIV and AIDS."*

The supplementary information in the *Retrovirology* article reports successful toxicology and pharmacokinetic studies.

Didier Scherrer, Ph.D., Vice-President of Small Molecules R&D at ABIVAX said, “As a result, ABIVAX has received authorization to carry out clinical studies with ABX464, following the phase I trial in human volunteers which was initiated in early 2014. Based on the safety and PK data, ABX464 progressed to a phase IIa trial in patients, which was begun several weeks ago. The goal of this study is to determine the optimal dose and frequency of administration.”

Professor Hartmut J. Ehrlich, M.D., CEO of ABIVAX, concluded, “We are very excited about the data obtained so far with ABX464, as they indicate that ABX464 is safe and may be less frequently administered than standard therapies, thereby potentially improving outcomes and compliance, helping to reduce healthcare costs and offering broader access to treatment.”

ABX464 is the first candidate molecule coming from ABIVAX’s proprietary anti-viral drug technology platform that focuses on viral RNAs. It has been generated from an in-depth understanding of the processing of viral RNA within the human host cell.

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## About ABIVAX

ABIVAX is an advanced clinical stage biotech company focused on becoming a global leader in the discovery, development and commercialization of anti-viral compounds and human vaccines to treat some of the world’s most important infectious diseases, including HIV/AIDS and chronic Hepatitis B.

ABIVAX has 2 compounds in clinical stage research: ABX464 a novel small molecule against HIV with a number of important potential competitive advantages, and ABX203, a therapeutic vaccine candidate that could be a cure for chronic hepatitis B. The broader ABIVAX portfolio includes additional anti-viral compounds and vaccines that may enter the clinical stage in the coming 18 months.

ABX464 has been developed using ABIVAX’ anti-viral platform that allows the Company to address a broad range of viral targets involved in the production and management of viral RNA within the host cell. ABIVAX also has access to a number of cutting edge technologies including complex molecular protein/RNA-pro interactions to discover and develop proprietary breakthrough therapies to help patients’ clear important pathogenic viruses.

Headquartered in Paris, France, ABIVAX conducts its research and development in Évry (France) and Montpellier (France). In addition, ABIVAX benefits from long term partnerships with the Cuban Center for Genetic Engineering and Biotechnology (Havana, Cuba), The Finlay Institute (Havana, Cuba), the Molecular Genetics Institute of Montpellier (CNRS-Université de Montpellier, France), the Curie Institute (Paris, France), the Scripps Research Institute (La Jolla, CA, USA), the University of Chicago (Chicago, IL, USA), Brigham Young University (Provo, UT, USA), and the Institut Pasteur (Paris, France). ABIVAX intends to pursue further business development opportunities to access commercial products as part of its overall corporate strategy.

ABIVAX was founded by Dr. Philippe Pouletty, M.D. , managing partner at Truffle Capital, the cornerstone investor in ABIVAX since its creation.

For more information, please visit the company’s website: [www.ABIVAX.com](http://www.ABIVAX.com)

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