



Innovative Medicines Initiative

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PRESS RELEASE

- **EU-AIMS – paving the way for new diagnostics and treatments in autism**

BRUSSELS, 6 May 2013 – IMI's EU-AIMS (European Autism Interventions - A Multicentre Study for Developing New Medications) project is making breakthroughs in research into autism spectrum disorder (ASD), that are set to make a real difference to the lives of people with autism and their families.

ASD affects around one in a hundred children, but still has no effective drug treatments. ASD symptoms include problems with social interactions, behaviour, co-ordination, language and communication. These range from mild to severe, and vary with age. Until recently, ASD was considered an untreatable condition. In EU-AIMS, teams of researchers from universities and industry, led by Roche and King's College London, along with patient groups, are working together to find out more about the causes of ASD, and to find new tests for the condition and identify potential drugs. By identifying children with ASD, or those who are most likely to develop it, and paving the way for new autism treatments, this project could change their lives and those of their families.

One of EU-AIMS's most exciting breakthroughs so far is a hint that it might be possible to reverse some of the brain changes in autism. The neuroligin-3 gene has been linked with inherited cases of autism. Mice that are missing the neuroligin-3 gene have overactive glutamate receptors and this causes problems with learning and brain development. When the mice are triggered to produce normal levels of neuroligin-3, the glutamate receptor activity returned to healthy levels, but more significantly, the autism-like changes in the mouse brains returned to normal.

Professor Declan G Murphy, MBBS, FRCPsych, MD, The Mortimer D Sackler Professor of Translational Neurodevelopment and Head of the Department of Forensic and Neurodevelopmental Sciences, King's College London, is the Academic Lead for the project. He commented: "We have used mouse models to identify new drug targets, providing new mechanisms for the disease that we didn't know a year ago. It's early days yet but if we can find the same abnormalities in humans, and can reverse them in the same way that we have reversed them in mice, we could slow the development of the disease and make it more manageable, or even prevent it completely. We have also demonstrated proof of concept that abnormal brain activity in adults with autism can be reversed by modulating brain serotonin. We now want to see if we can translate those findings to the clinic."

EU-AIMS will also work on the two largest ever clinical studies of ASD. The first study will look at the risk of autism in a younger sibling of a child with autism, while the second will track how symptoms change with age. These will involve around a thousand patients and will kick off in 2014.

Project Coordinator Will Spooren, Head Behavioural Pharmacology and Preclinical Imaging at Hoffmann-La Roche, explained: "We don't yet know the natural course of autism, as the symptoms differ at different ages. These studies will help us understand risk factors and learn about the needs of people and their families at different stages."

A down to earth and practical outcome of the collaboration is the project's contribution to the development of new treatment guidelines that are being put together by the European Medicines Agency. The guidelines will be available over the next 18 months or two years. Other key achievements of the project so far include finding out more about why the age of parents affects the chance of children having ASD, finding links between pathways in different mice with autism symptoms,

To help support research, the collaboration has generated the first line of induced pluripotent stem cells (iPS cells) from skin cells from autism patients so that researchers can understand the differences between healthy people and autism patients, and has created a repository of tissue and blood samples from

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animals and patients.

This work is part of the Innovative Medicines Initiative (IMI), a collaboration between the European Union and the pharmaceutical industry. By supporting the exchange of knowledge and expertise among companies and between public and private partners, IMI is generating achievements and taking on research challenges that are too great for any individual company or organisation to tackle alone. The ultimate goal of IMI is to speed up the development of safer and more effective medicines for patients.

IMI Executive Director Michel Goldman said: 'EU-AIMS demonstrates that by bringing together top experts from the public and private sectors, it is possible to make great advances in our understanding of autism and deliver results that will pave the way for new, more effective treatments for the condition.'

More information:

Project factsheet: <http://www.imi.europa.eu/content/eu-aims>

Project website: <http://www.eu-aims.eu/>

- **Interview opportunities will be available at the [IMI Stakeholder Forum](#) in Brussels on 13 May 2013.**

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About the EU-AIMS project

Around 1% of children are diagnosed with autism spectrum disorders (ASD), yet there are currently no drugs designed specifically to treat their main symptoms. Working to change this is the IMI-funded EU-AIMS project. The goal of EU-AIMS is to generate tools that will enhance our understanding of ASD, and ultimately pave the way for the development of new, safe, and effective treatments for use in both children and adults. As well as dramatically improving quality of life, good treatments would help to cut the social and economic costs of ASD.

About IMI

The Innovative Medicines Initiative (IMI) is the world's largest public-private partnership in health care. IMI is improving the environment for pharmaceutical innovation in Europe by engaging and supporting networks of industrial and academic experts in collaborative research projects. The European Union contributes €1 billion to the IMI research programme, which is matched by in kind contributions worth at least another €1 billion from the member companies of the European Federation of Pharmaceutical Industries and Associations (EFPIA).

The Innovative Medicines Initiative currently supports 40 projects, many of which are already producing impressive results. The projects are all working to address the biggest challenges in drug development, to accelerate the development of safer and more effective treatments for patients.

More info: www.imi.europa.eu

