



## SCIENTIFIC WEBINAR

Tuesday 24<sup>th</sup> May 2022

16.00 CEST / 10.00 EDT / 07.00 PDT

# Drugging the RNA World

### Speakers



**Stefan Lohmer, PhD**  
CEO & CSO



**Lia Scarabottolo, PhD**  
Director, Discovery Services

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# Webinar

## Summary

The recurrent problematic clinical delivery of oligonucleotide-based therapies but also the clinical success of Risdiplam, a small molecule-based RNA splicing modulator, has led to a very strong interest in small molecules targeting the more complex RNA structures and/or the dynamic landscape of RNA. Importantly, RNA molecules have stable tertiary, and in some cases, quaternary structures. These structural features offer new opportunities for selective RNA targeting with small molecules, that could lead to a tailor made modification of RNA functions or alter gene expression and cellular processes towards the control of disease.

During this webinar, we will show you how Axxam uses its HTS/HCS platforms to identify small molecules drugging RNA, at the cellular and subcellular levels, in miniaturized high-throughput formats. This journey will encompass automated TaqMan™-based HTS for the detection of molecules modulating RNA transcription or RNA splicing, as well as the use of innovative smart assays, like mini-genes or the use of RNA-protein interaction assays, keeping a focus on the relevant human disease.

**Key words:** Splicing modulators; promotor activation/silencing; transcription factor; Riboswitch; RiboTAC; shRN; t-RNA modification; TaqMan™-based HTS; mini genes; translocation assays; RNA-Protein interactions; epitranscriptomics.

## About the speakers

Stefan Lohmer is Axxam's co-founder, Chairman of the Board of Directors and Chief Executive Officer. Prior to founding Axxam, he was head of the assay development unit at the Bayer Research Centre in Milan and the Head of Genomics worldwide for Bayer AG. He was responsible for generating and managing Bayer's external genomic alliances with Millennium and Lion Bioscience. Stefan Lohmer joined Bayer in 1992. He holds a degree in Molecular Biology and Biochemistry from the University of Cologne and completed his PhD at the Max Planck Institute for Plant Breeding in Cologne.

Lia Scarabottolo has a degree in Pharmaceutical Chemistry and Technology and a Master in Experimental Pharmacology (University of Milan). Since January 2007, she is Director of the Discovery Services Department at AXXAM, with the responsibility of the units of: Cell Biology, Biochemistry, Screening Technologies, Electrophysiology, High Content Screening, Molecular Biology, Cell Factory and iPSC. She is program director for many of the collaborations in place with national and international companies, as well as scientific coordinator for projects granted by the Italian government, the European Commission, and international organisms.

**Please note:** This is a free webinar and places are limited. We reserve the right to turn down any registrant. After registering you will receive a confirmation email containing a link to access the webinar.

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