STORM THERAPEUTICS ANNOUNCES PUBLICATION IN NATURE ON RNA EPIGENETICS BY FOUNDER PROFESSOR TONY KOUZARIDES

New Drug Target Discovered For Acute Myeloid Leukaemia

27th November 2017, Cambridge, UK: STORM Therapeutics, the leading drug discovery company focused on the discovery of small molecule therapies modulating RNA epigenetics, today announced the publication of data in the internationally renowned scientific journal ***Nature*** linking an essential RNA-modifying enzyme to acute myeloid leukaemia (AML)**.**

The study has found an unexpected new drug target for acute myeloid leukaemia (AML) that could open new avenues to develop effective treatments against this potentially lethal disease. Data show that inhibiting the *METTL3* gene destroys human and mouse AML cells without harming non-leukaemic blood cells. The paper, entitled "*Promoter-bound METTL3 maintains myeloid leukaemia via m6A-dependent translation control'*, goes on to reveal why *METTL3* is required for AML cell survival, by deciphering the new mechanism it uses to regulate several other leukaemia genes.

Professor Tony Kouzarides, Founder of STORM Therapeutics and joint project leader from the Gurdon Institute, University of Cambridge, commented: "This is an important milestone in the understanding of RNA epigenetics and its links to disease. These findings highlight the importance of RNA modifying enzymes in cancer, and in particular in leukaemia. New treatments for AML are desperately needed and we have been looking for novel genes that would be good drug targets. We identified the methyl transferase enzyme METTL3 as a highly viable target against AML. Our study will inspire pharmaceutical efforts to find drugs that specifically inhibit METTL3 to treat AML."

STORM has established a pipeline of drug discovery programmes to develop novel, first-in-class drugs for the treatment of specific cancers and other diseases with high unmet medical need. It is focusing on two classes of RNA modifying enzymes, RNA methyltransferases and terminal uridyltransferases (TUTases), and has already advanced two undisclosed targets in drug discovery.

Keith Blundy, CEO of STORM Therapeutics, said: "STORM leads the field of harnessing the power of RNA epigenetics as a new area of important biology. Our ambition is to become a world leading therapeutics company tackling diseases through modulating RNA modifying enzymes. Publication of these data, in such a prestigious journal as Nature, is validation of the world class science on which the Company was founded."

This publication represents results obtained from research conducted in collaboration by: Wellcome Trust Sanger Institute, The Gurdon Institute and Department of Pathology at the University of Cambridge, Cold Spring Harbor Laboratory and STORM Therapeutics.

Publication:

Isaia Barbieri, Konstantinos Tzelepis , Luca Pandolfini , Alan Hendrick *et al.* Promoter-bound METTL3 maintains myeloid leukaemiaby m6A-dependent translation control. *Nature*. DOI: 10.1038/nature24678

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NOTES TO EDITORS

About STORM

STORM Therapeutics is a University of Cambridge spin-out, translating the groundbreaking work of Professors Tony Kouzarides and Eric Miska in RNA epigenetics into the discovery of first-in-class drugs in oncology and other diseases. It is the leading company currently tackling disease through modulating RNA modifying enzymes and is developing a unique platform to address these enzyme classes, including RNA methyltransferases. STORM is backed by blue chip investors Cambridge Innovation Capital, Merck Ventures, Pfizer Ventures and Touchstone Innovations, who share the founders' ambitions to build a world-leading company in the field. The company raised its series A funding in June 2016 and occupies modern, well-equipped laboratories on the Babraham Research Campus near Cambridge, UK. For more information, please visit http://www.stormtherapeutics.com/