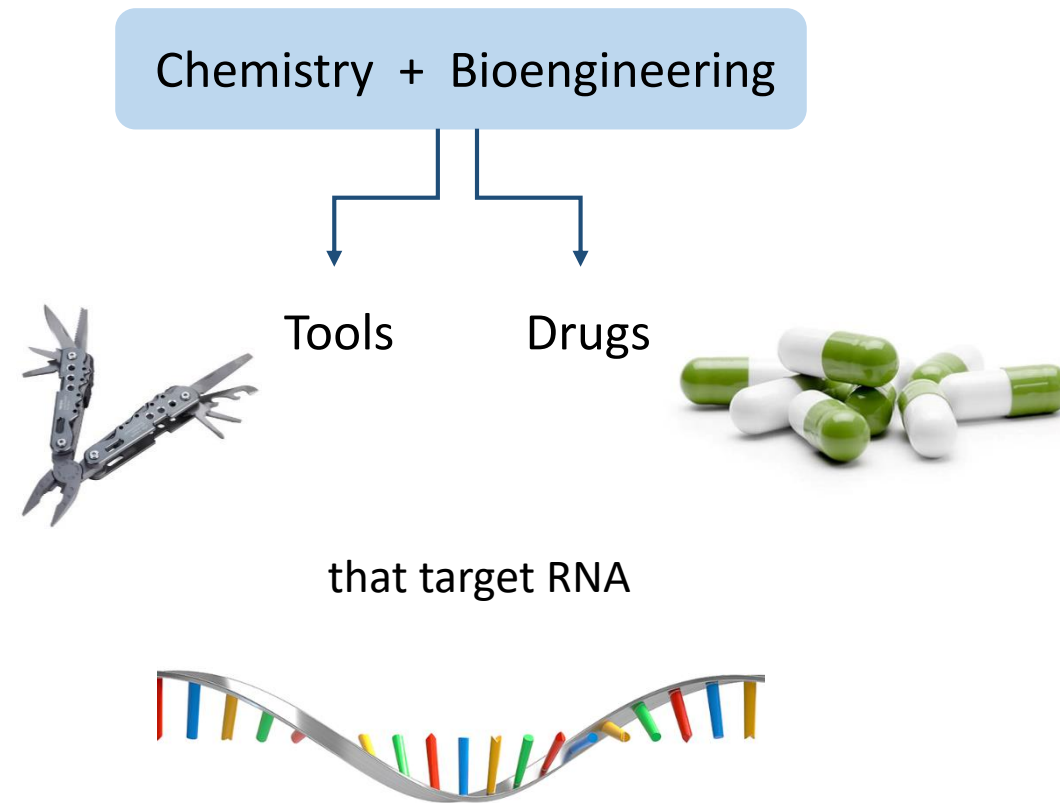




Site-directed RNA base editing – A marketable innovation





Site-directed RNA base editing

Chemistry + Bioengineering

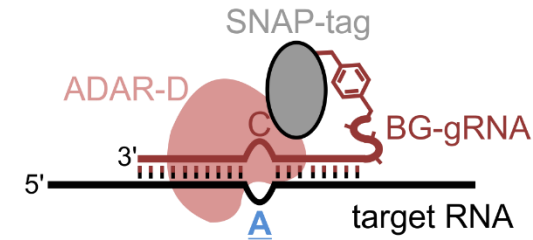
Tools

Drugs

that target RNA



SNAP-ADAR

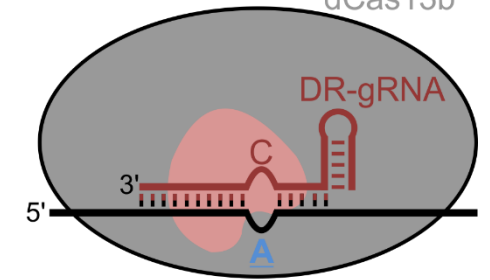


Stafforst

ACIE 2012

Nat. Meth. 2018

dCas13b-ADAR



Zhang Science 2017





Site-directed RNA base editing

Chemistry + Bioengineering

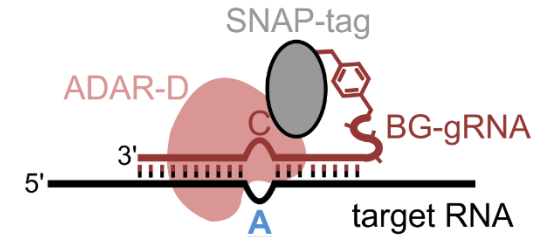
Tools

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SNAP-ADAR

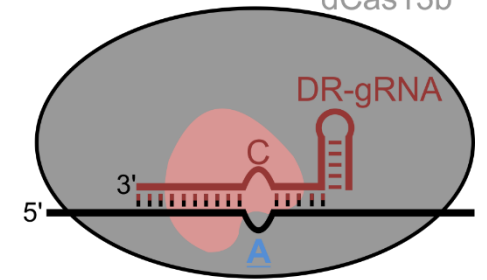


Stafforst

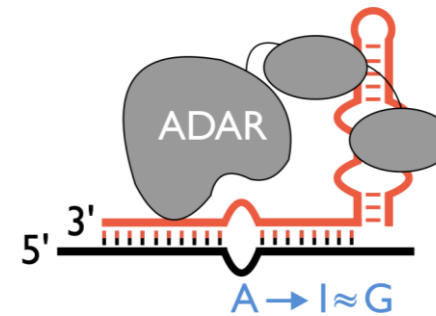
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Stafforst Nat. Biotech. 2019

RESTORE

- chem. mod. ASO
 - harness endog. ADAR
 - highly precise A-to-I
 - novel drug platform
 - dosable, reversible
- safer, patient compliance (?)



The promise of therapeutic RNA base editing

☰
c&en
👤

Watch out, CRISPR. The RNA editing race is on

Start-ups and academic labs are des...
precisely edit RNA

by [Ryan Cross](#)

MARCH 25, 2019 | APPEARED IN **VOLUME 97, ISSUE 12**

▼ **nature**

NEWS FEATURE · 04 FEBRUARY 2020

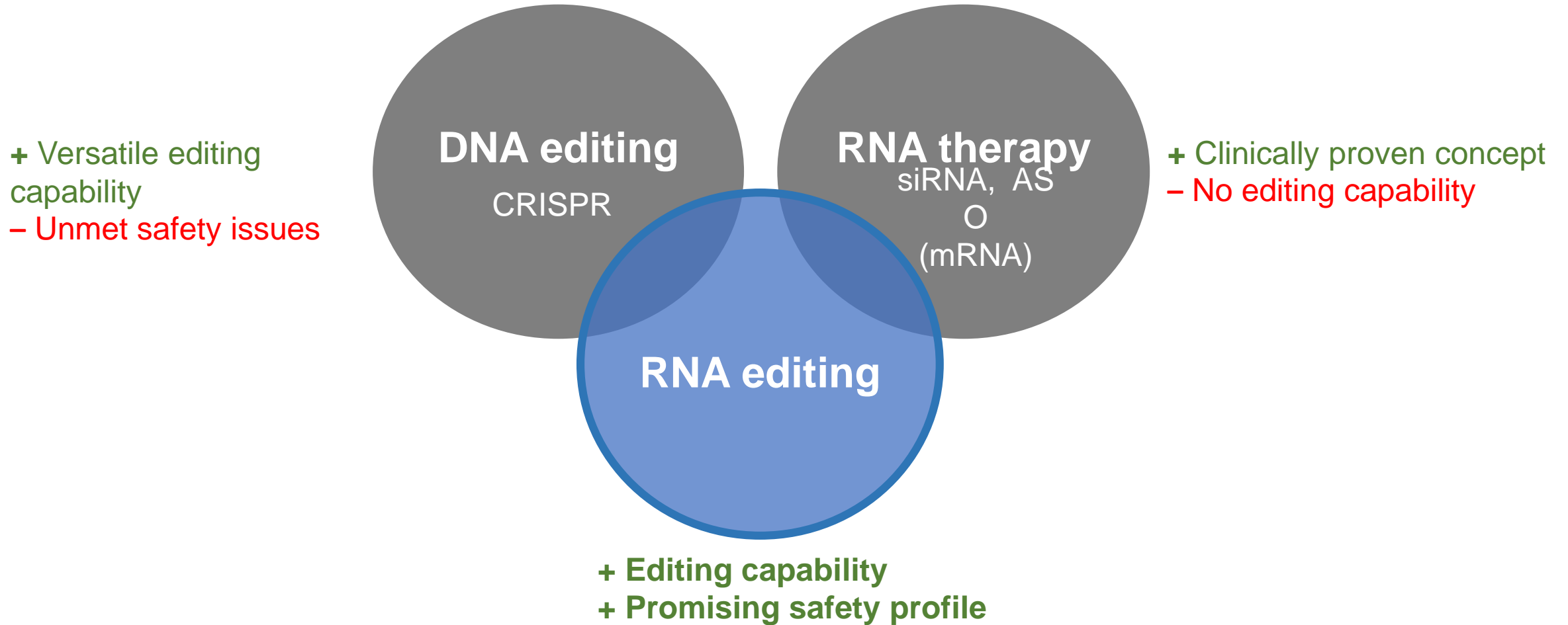
Step aside CRISPR, RNA editing is taking off

Making changes to the molecular messengers that create proteins might offer flexible therapies for cancer, pain or high cholesterol, in addition to genetic disorders.

Sara Reardon



The promise of therapeutic RNA base editing





Bottlenecks Knowledge → Innovation (EU)

(Young) Academic Researcher

- lacking experience
 - approach investor – when&how?
 - start-up – when&how?
 - patent or publish?

Processes at Academic Institutions

- slow patenting
- no patenting strategy
- little reward for patents/IP
- little interest in translation
 - e.g. consulting, sponsored research, licensing, lab space → Col discussion
 - almost no Incubators for Biotech

Investors

- don't stay in US come to EU
- make use of ideas and heads
- talk to academics early
- fund novel ideas early (Incubator)

EU

- yearly seed funding program (2-3 Mio EUR/project)

Academic System (Paper, Grants, Positions)

- teaching/administrative burden
- Grants & PhD project (low risk – fast gain)
 - too short-termed (3y)
 - too structured
- high publ. frequency expected
- fashionable vs. sustained/novel directions