

## RNA標的の低分子開発研究の社会的要請、学術的意義は極めて高い

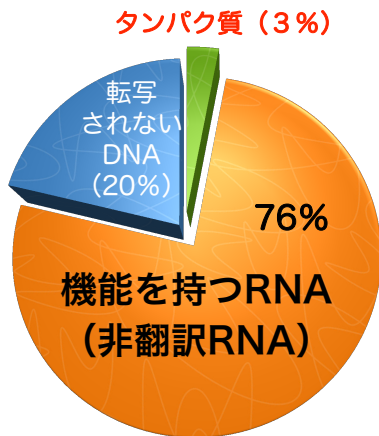
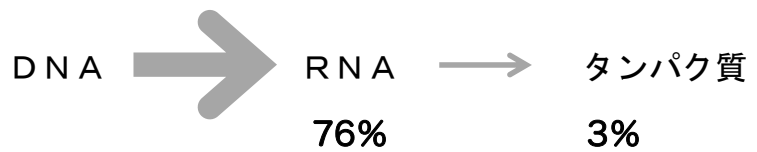


図1 ヒトゲノム30億塩基対の行方

ゲノム解析 以前の考え方



ゲノム解析 以後に明らかに



- ・ ヒトゲノムはほんの一部しかタンパク質に翻訳されない
- ・ 大半は、RNAに転写されておしまい。
- ・ ゲノム解析から10年経ち、RNAに機能があることが明らかに。
- ・ 創薬では、タンパク質に加えて、RNAを標的とすることが必須

## アメリカ企業はどんどん先へ。例えば、

**c&en**  
CHEMICAL & ENGINEERING NEWS

Latest News  
Web Date: February 27, 2017

Arrakis launches to develop RNA-targeting small-molecule drugs

Biotech firm will chase an array of previously intractable targets  
By Lisa M Jarvis

With \$38 million in funding from a swath of investors, Arrakis Therapeutics has formally launched to create a new class of drugs: small molecules that directly bind to RNA.



Arrakis Therapeutics  
\$3800万ドルを調達

**ribometrix**

Small molecule against RNA



RIBOMETRIX  
\$750万ドルを調達



Press Release

Expansion Therapeutics  
Raises \$55.3 Million in Series A Financing to Advance Portfolio of Novel RNA Targeted Small Molecule Medicines to Treat Rare Diseases

Expansion Therapeutics  
\$5500万ドルを調達



## 日本における主な取り組み

### 大学・研究機関

- ・大阪大学 産業科学研究所 中谷研究室  
核酸標的的低分子の開発（遺伝子疾患関連配列ほか）
- ・千葉工業大学 河合剛太 教授  
核酸—低分子複合体の構造解析

### 企業 ほか

- ・（財）大阪大学産業科学研究協会 新産業創造研究会  
核酸標的的低分子創薬研究会 2017.4～  
代表 中谷和彦
- ・NPO法人 mRNAターゲット創薬研究機構 2017.4～  
理事長 河合剛太、副理事長 中谷和彦

Chemical & Engineering News (アメリカ化学会広報誌), 2017 November 27.

**Features**

DRUG DISCOVERY

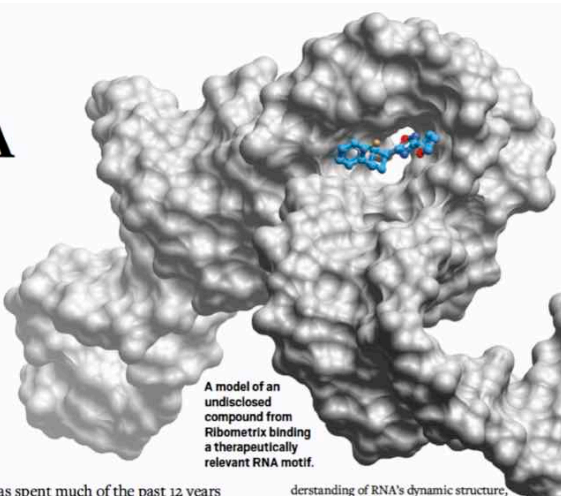
## The RNA drug hunters

Academics, biotech start-ups, and big pharma companies are designing small molecules that target RNA

RYAN CROSS, C&EN BOSTON

**M**atthew Disney has spent much of the past 12 years countering doubtful, sometimes even dismissive, remarks about his work. Fellow academic researchers said his science was too risky; federal agencies were reluctant to fund it. His friends in industry said there simply weren't enough data.

understanding of RNA's dynamic structure, the expanding number of RNAs linked to chronic diseases, and the bravado of early researchers have motivated at least a dozen pharma and biotech companies to dedicate themselves to the task.



A model of an undisclosed compound from Ribometrix binding a therapeutically relevant RNA motif.

### RNA gold rush

At least a dozen companies have programs dedicated to finding small molecule drugs that target RNA.

COMPANY	PROGRAMS
Arrakis	Huntington's disease, undruggable proteins, noncoding RNA
Biogen	Spinal muscular atrophy
Boehringer Ingelheim	Undisclosed programs
Expansion Therapeutics	Rare genetic diseases
Merck & Co.	Riboswitches (previously) Noncoding RNA (currently)
Novartis	Spinal muscular atrophy Undisclosed programs
Novation Pharmaceuticals	Inflammation, psoriasis
Nymrum	Undisclosed programs
Pfizer	Undisclosed programs
Ribometrix	Undruggable proteins and noncoding RNA
TargetRNA	Non-coding RNA
The RNA Medicines Co.	microRNA in triple negative breast cancer and glioblastoma