

New therapies for Parkinson's – what's going on?

Kevin McFarthing

3 January 2018

Tonight.....

- Numbers of projects
- What are researchers trying to do?
- What will 2018 bring?
- What else is exciting?

Me.....

- Biochemist
- Research products
- Diagnostics
- Pharmaceuticals
- Consumer products



How many Parkinson's therapies are
in research and development?

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in research and development?

200*

*at least....

DISEASE MODIFYING



RELIEVE

symptoms

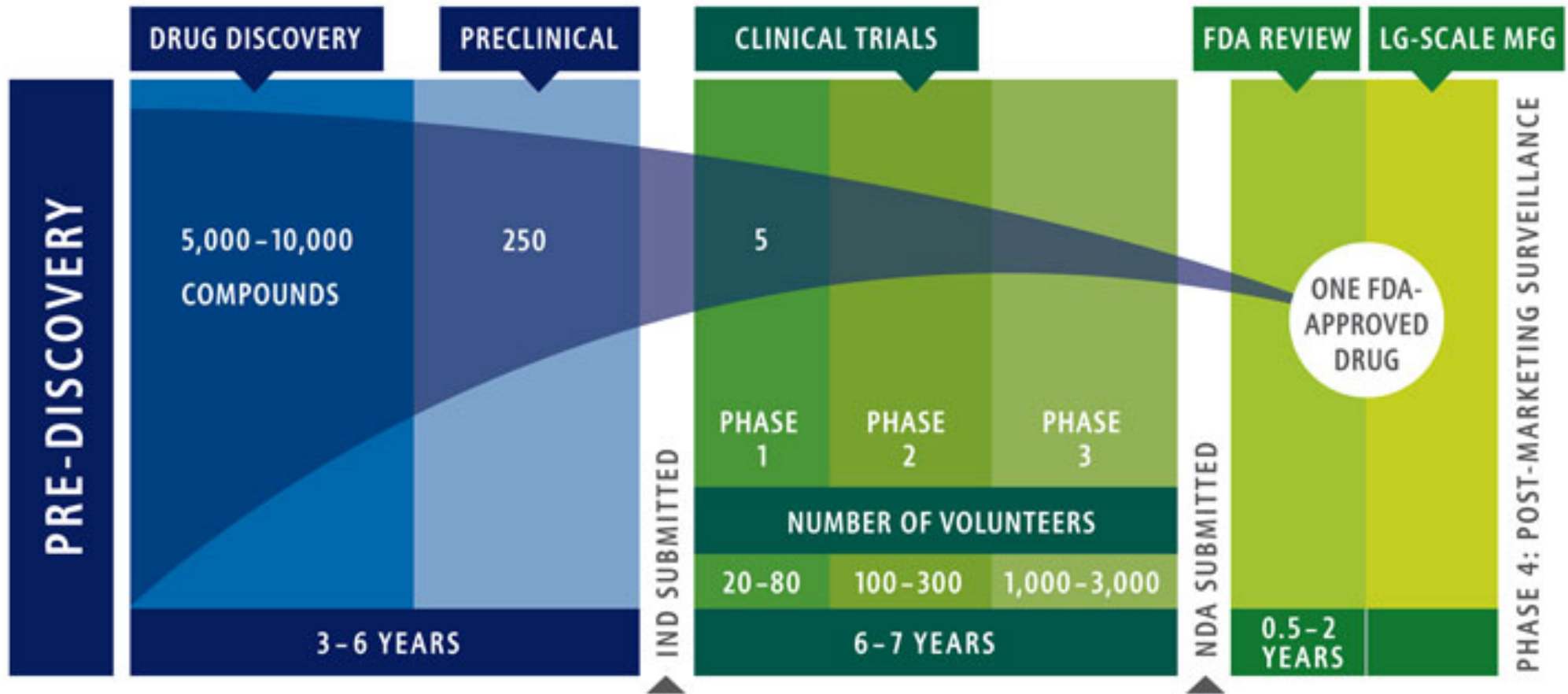
RETARD

disease progress

REPLACE

what's been lost

Drug Discovery and Development: A LONG, RISKY ROAD



Source: Pharmaceutical Research and Manufacturers of America

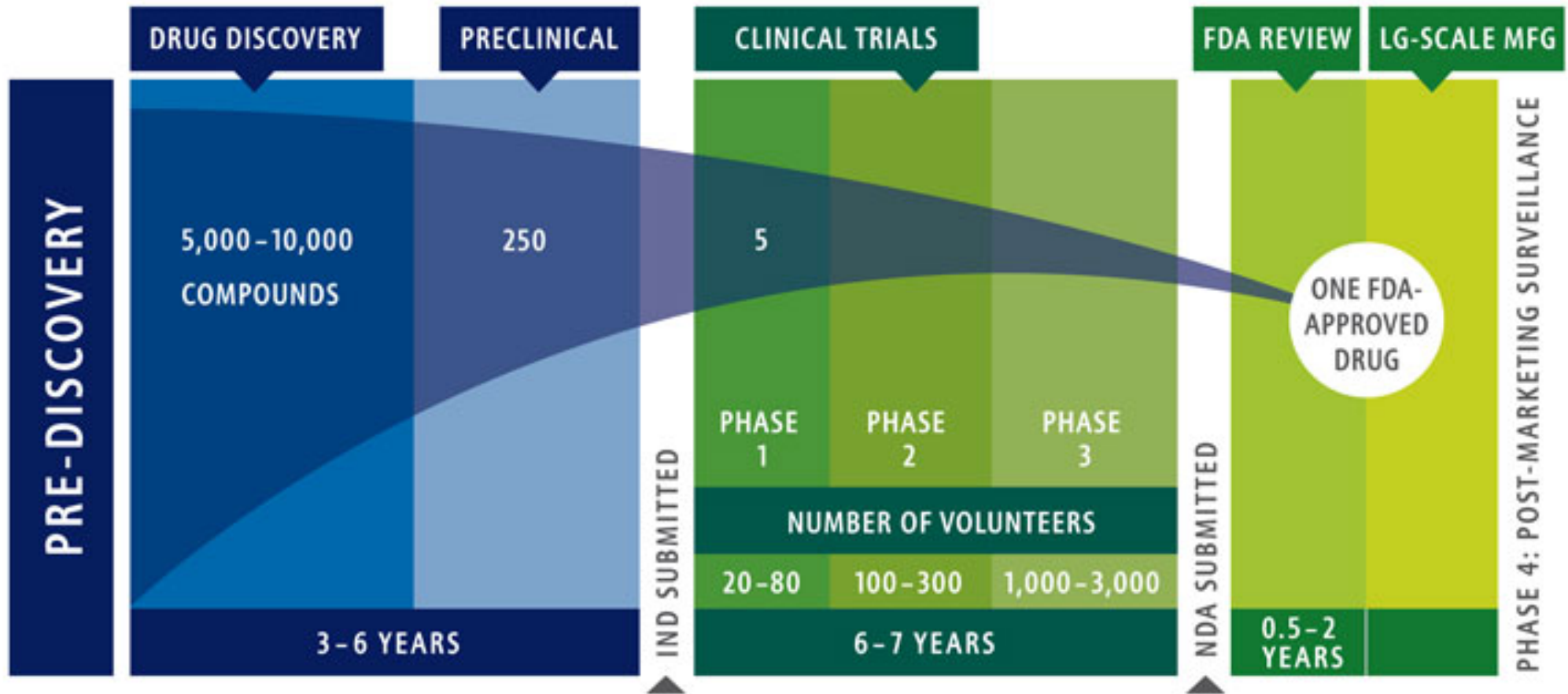
Some glossary

- **NCE** – new chemical entity; a molecule that is new to the pharmaceutical world
- **Reformulation** – a new way to deliver the same drug; for example, a tablet to a transdermal patch
- **Repurpose** – a drug already used in one disease re-tested for another; for example, diabetes to Parkinson's
- **Cells** – injection of, for example, stem cells to replace dead cells
- **Gene therapy** – delivering DNA to replace what's been lost

What's different about clinical trials for Parkinson's?

- We're all different
- Genetic factors
- Measuring impact on disease progression is very difficult
- Relative lack of objective measurements
- High placebo response
- Selection bias

Drug Discovery and Development: A LONG, RISKY ROAD



Source: Pharmaceutical Research and Manufacturers of America

Summary

RESEARCH/PRE-CLINICAL		CLINICAL	
Discovery	49	Phase 1	29
Pre-clinical	56	Phase 2	43
Research program	7	Phase 3	7
		Proof of concept	7
		Submitted	2
TOTAL	112	TOTAL	88
Symptom relief	21		
Disease modifying	89	Symptom relief	48
Not clear	2	Disease modifying	40
TOTAL	112	TOTAL	88
NCE	93	NCE	46
Reformulation	5	Reformulation	15
Repurpose	8	Repurpose	20
Cells	5	Cells	3
Gene therapy	1	Non-invasive surgery	1
		Gene therapy	3
TOTAL	112	TOTAL	88

What will 2018 deliver?

RELIEVE

symptoms

RETARD

disease progress

REPLACE

what's been lost

New to the market therapies

RELIEVE

symptoms

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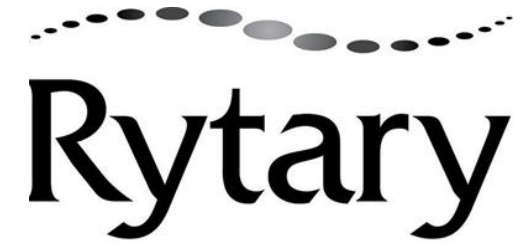
Gocovri

RETARD

disease progress

REPLACE

what's been lost



- Extended release carbidopa and levodopa
- Significantly less OFF time and more ON time without dyskinesia
- Approved in the USA
 - \$250 per 90 capsules vs \$17 for Sinemet vs \$35 for Sinemet CR
- US focus at the moment



- Extended release amantadine capsules for the treatment of dyskinesia
- Additional 3.6 hrs without troublesome DKs
- About one hour less OFF time
- Approved in the USA
 - \$28,500 per year
- US focus at the moment



INBRIJA

- Inhaled L-DOPA
- Starts to work within 10 minutes
- Rejected by FDA in July 2017
- Resubmitted in December

Phase 3 trials underway

RELIEVE

symptoms

RETARD

disease progress

REPLACE

what's been lost

Tozadenant.....

L-DOPA extended release

Apomorphine thin film

Ropinirole patch

Rasagiline/pramiprexole combo



Tozadenant

FierceBiotech

BIOTECH RESEARCH IT CRO MEDTECH

Biotech

Acorda dumps \$363M Parkinson's drug after patient deaths

by Nick Paul Taylor | Nov 20, 2017 8:34am

Phase 3 trials underway

RELIEVE

symptoms

RETARD

disease progress

REPLACE

what's been lost

Isradipine

Inosine

Deferiprone

What's exciting? Drugs "repurposed" from...

RELIEVE

symptoms

Cough relief
Thalassemia
High blood pressure
Anti-anxiety
Athletic performance
Antispasmodic
Antifungal
Sedative

RETARD

disease progress

Diabetes
Cholesterol lowering
Anti-oxidant
Smoking cessation
Leukemia

REPLACE

what's been lost

What's exciting?

RELIEVE

symptoms

Faster acting

Longer lasting

Less dyskinesia

RETARD

disease progress

Immunotherapy

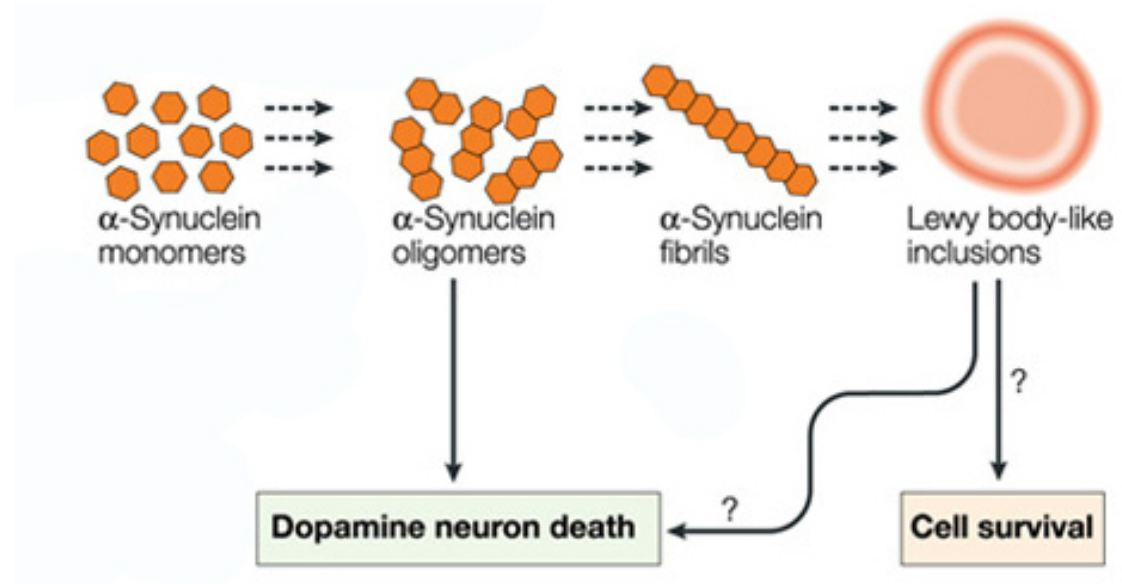
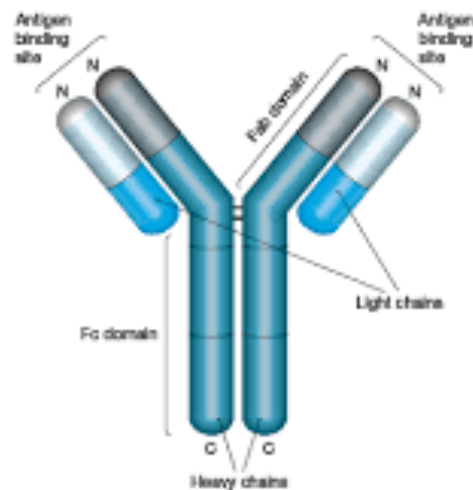
Correct problems with
mitochondria

Gene therapy

REPLACE

what's been lost

Stem cells



PHASE 1

Affiris – peptide mimic vaccine

Astra Zeneca and Takeda – antibody

Biogen – antibody

Neuropore/UCB – inhibits aSN aggregation

PHASE 2

Prothena/Roche - antibody

Enterin – displaces aSN aggregates

QR Pharma – inhibits aSN aggregation

Sanofi/Genzyme – inhibits aSN aggregation

Images: BioRad and ScienceofParkinsons.com

Denali pulls off 2017's biggest biotech IPO, pocketing \$250M for Parkinson's, Alzheimer's trials

by Nick Paul Taylor | Dec 8, 2017 9:13am

GEN News Highlights

[More](#)

July 24, 2017

Mitsubishi Tanabe to Buy Parkinson's Disease Drugs Firm NeuroDerm for \$1.1B

Conclusions

- There really is a lot going on
- Parkinson's is an attractive “market”
- It isn't easy
- It isn't cheap
- There are some very large companies involved
- The investment is very large

Thank you!!

Backup slides



- Extended release L-DOPA
- “Accordion Pill”
- ACCORDANCE phase 3 trial should report in 2018
- <https://www.youtube.com/watch?v=1qJeFrwkonc#action=share>



- APL-130277
- Apomorphine sub-lingual thin film for treatment of OFF time
- Phase 3 studies were planned to finish in 2017
- Interim results were positive
- Originally planned to apply for FDA approval end of 2017
- Should report in 2018





- HP-3000
- Ropinirole skin patch
- Due to file for approval in Japan in 2018

P2B001 – pramiprexole/rasagiline combo

DDF 2017 FiercePharma Jobs Resources

FierceBiotech

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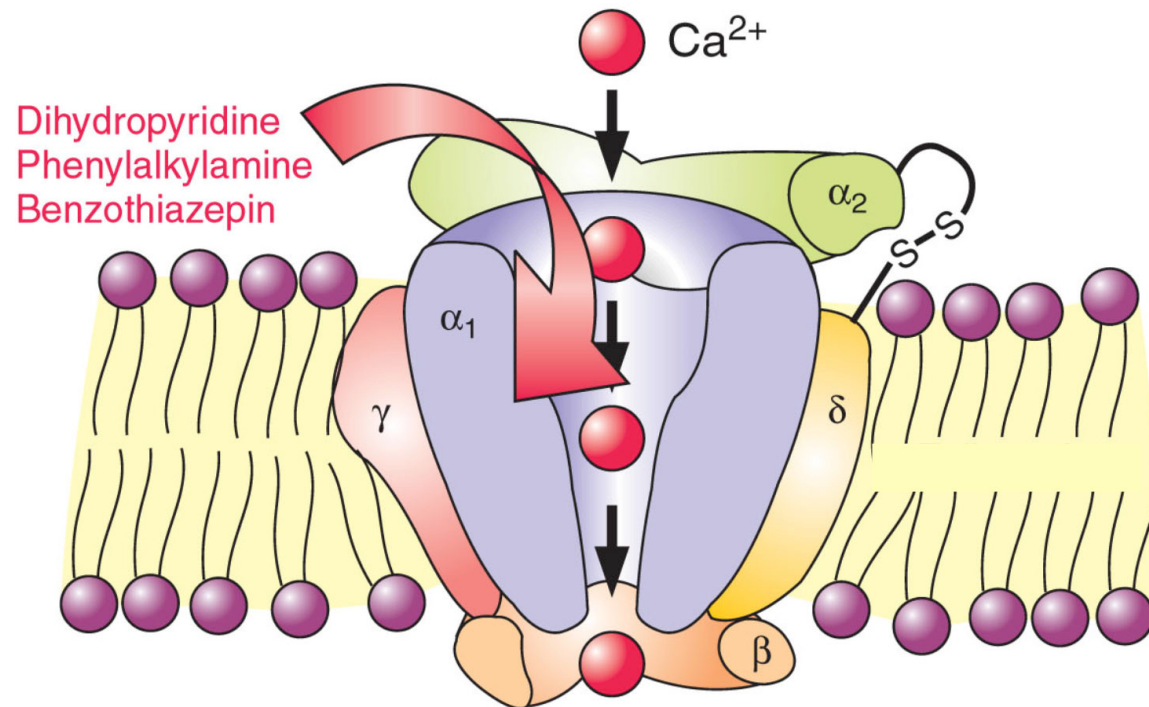
Venture Capital

Pharma Two B raises \$30M for Parkinson's phase 3 from Biogen-backed fund, adds ex-Pfizer CEO to board

by Nick Paul Taylor | Feb 27, 2017 7:34am

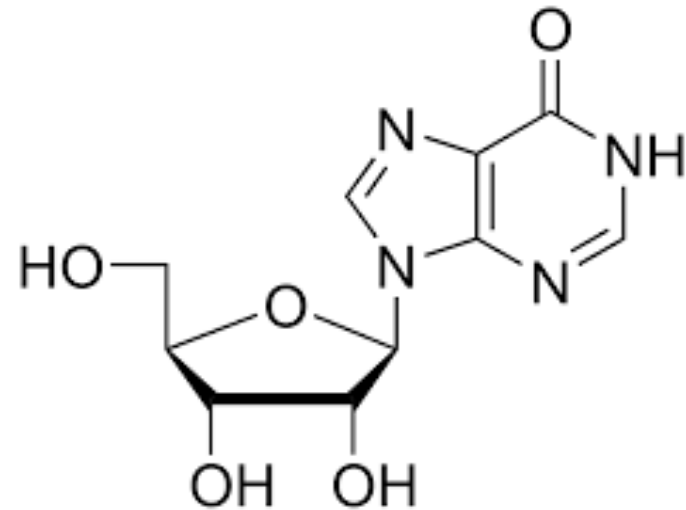
Isradipine

- Calcium channel blocker
- Already in use (Dynacirc) for high blood pressure
- Evidence to suggest slowing of progression
- Phase 3 study reports in 2019



Inosine

- Levels of uric acid associated with lower risk of PD
- PD appears to progress more slowly in people with higher levels of uric acid
- Inosine is a precursor of uric acid
- Phase 3 trial under way, due to report in 2020



Deferiprone

- Iron chelator
- On the market since 1999
- Phase 3 due to report in July 2018



The Huntington's Disease breakthrough

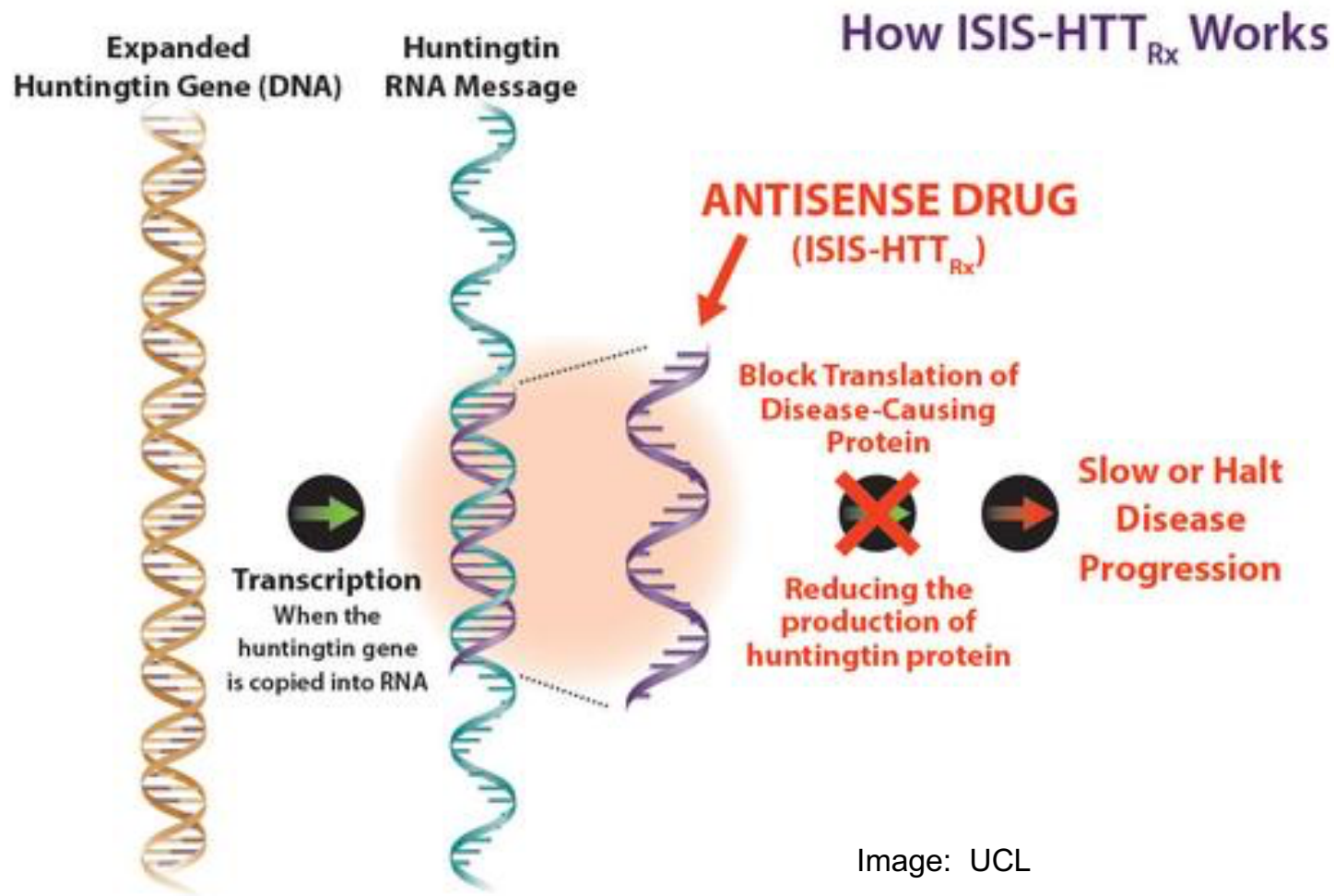


Image: UCL