

The logo features a large, stylized '10C' in white. The '10' is composed of three vertical bars of varying heights, and the 'C' is a thick, open circle. The text '10° ENCUENTRO CIBIC 2019' is centered within the 'C' in a bold, white, sans-serif font.

**10° ENCUENTRO
CIBIC 2019**

10 AÑOS
COMPARTIENDO
INNOVACIÓN

Desde la edición génica hasta el diagnóstico: usos de CRISPR-Cas en biomedicina

Dr. Pereyra Bonnet Federico

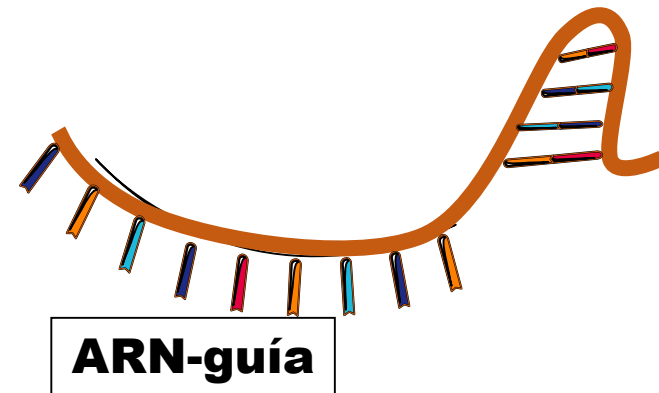
INPA-CONICET-Universidad de Buenos Aires



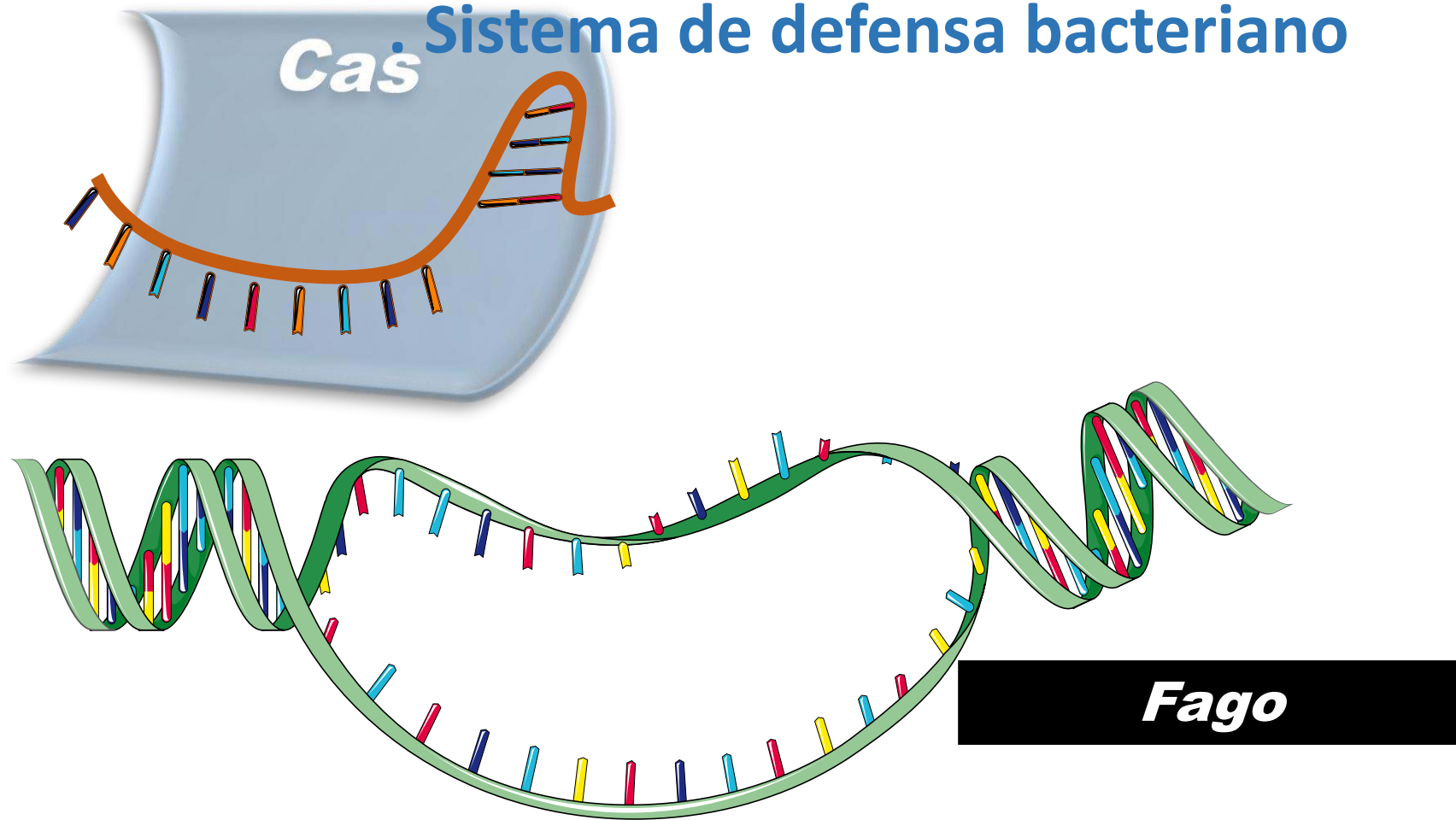


. El Origen

. Sistema de defensa bacteriano

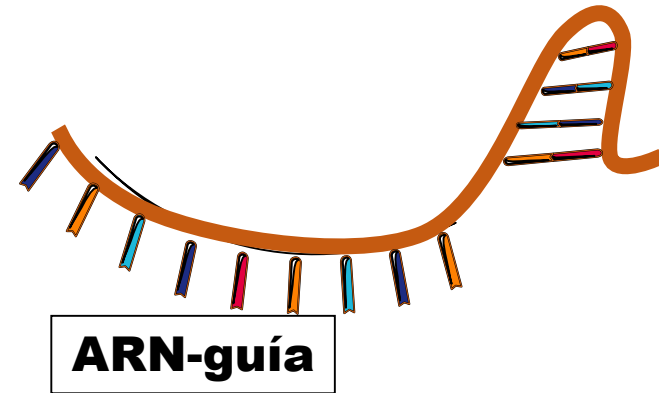


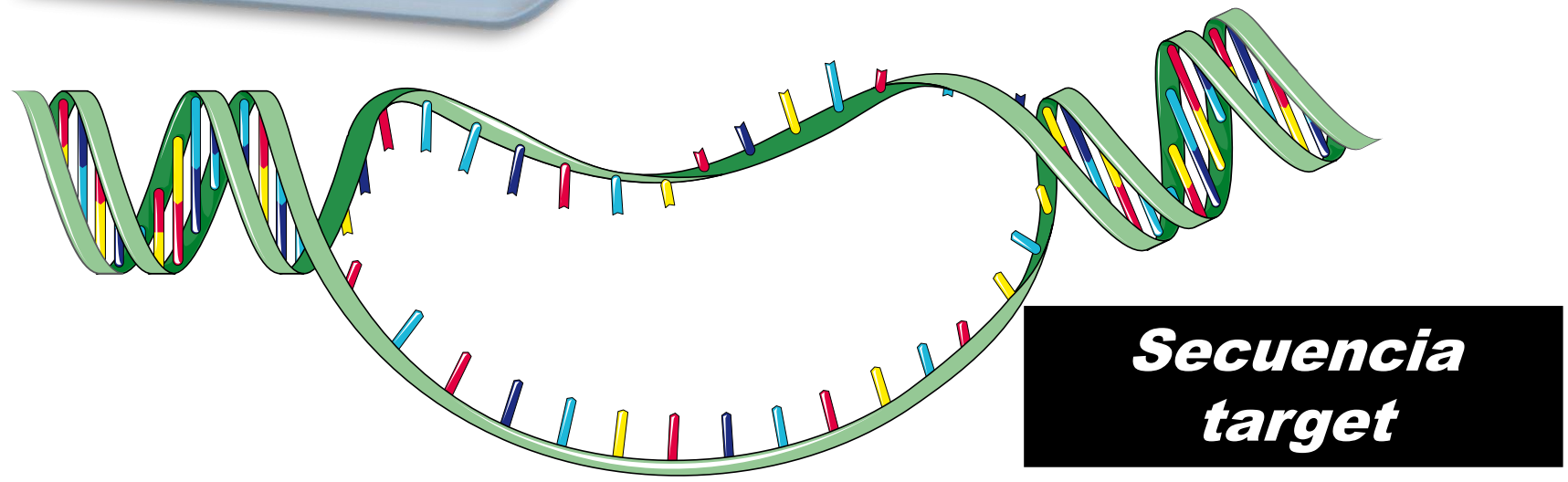
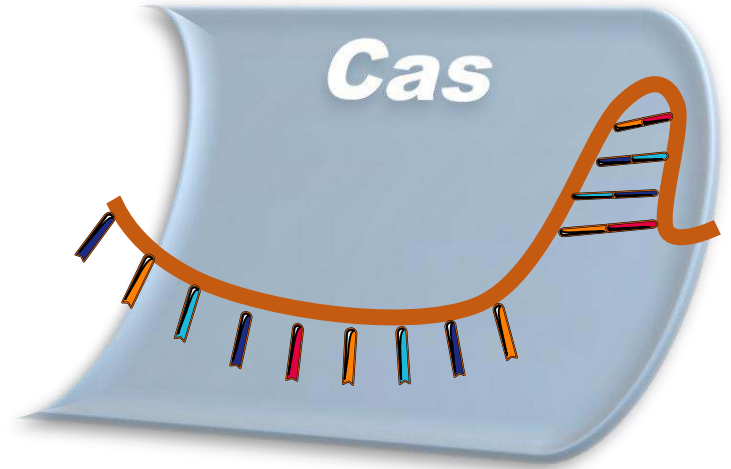
Sistema de defensa bacteriano





. Edición Génica



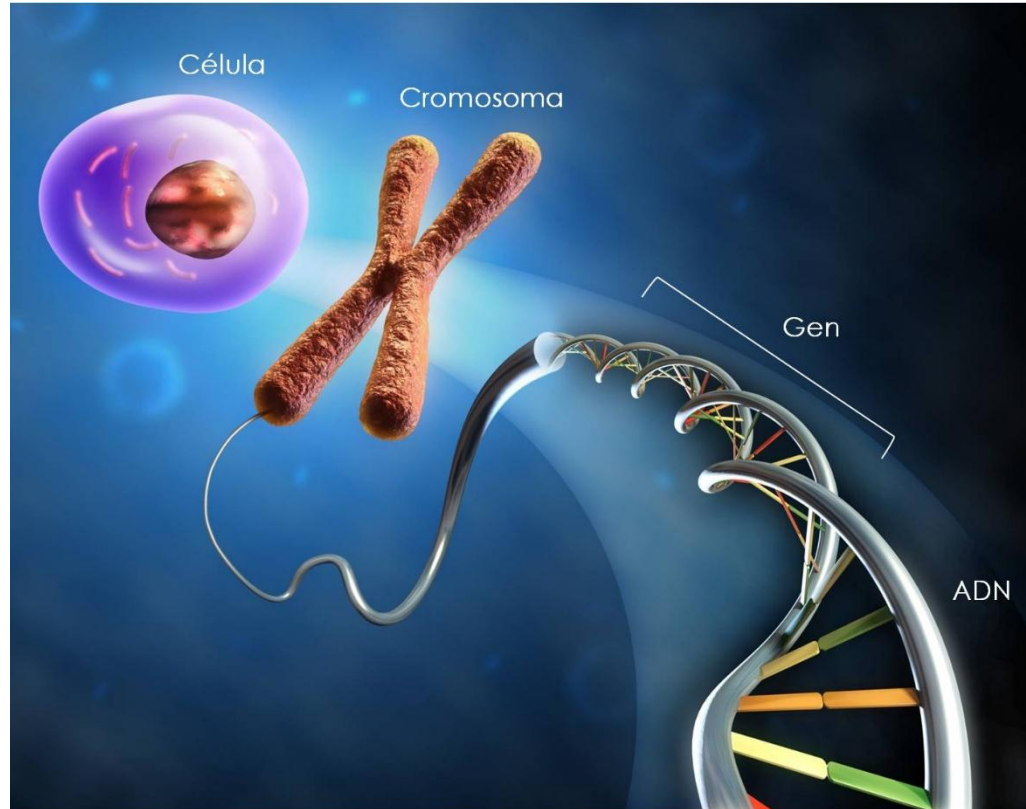


Síndrome de Treacher Collins



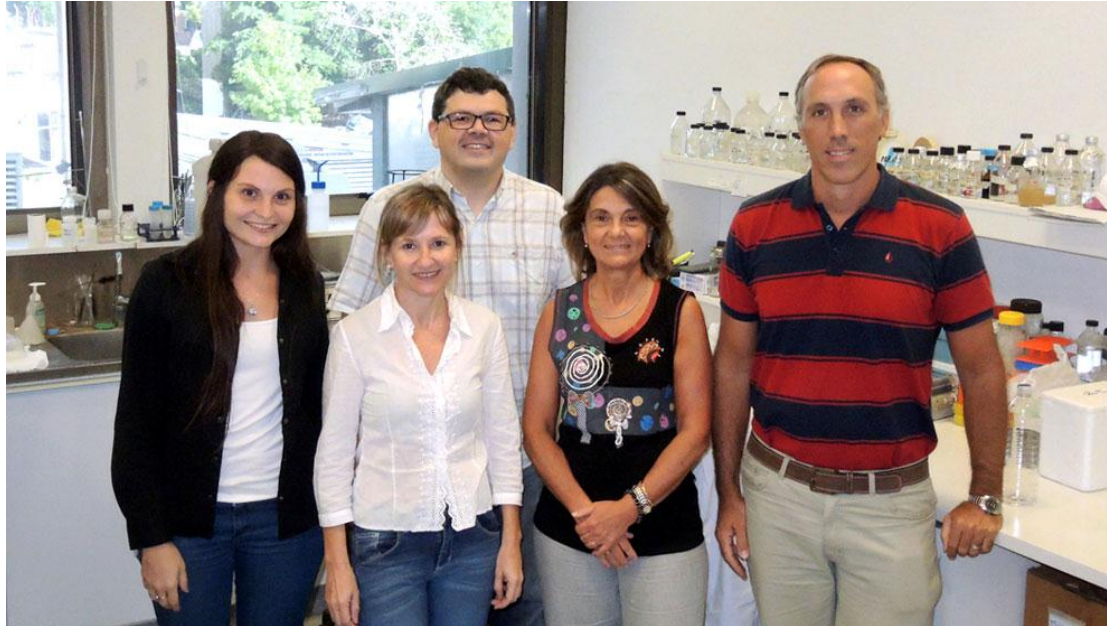
- ❖ Auggie Pullman es el niño de la película “Extraordinario” que tiene el síndrome de Treacher Collins.
- ❖ Es una enfermedad genética caracterizada por malformaciones craneofaciales (1/50000).
- ❖ Al día de hoy sólo se pueden corregir las malformaciones mediante cirugía reconstructiva.

Síndrome de Treacher Collins



En esta enfermedad, está mutado el gen *TCOF1* localizado en el cromosoma 5

Síndrome de Treacher Collins

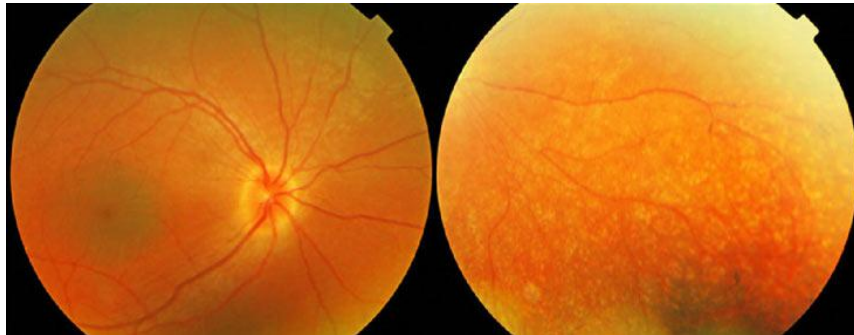


- ❖ La Dra Nora Calcaterra y equipo están editando el gen *TCOF1* en un modelo animal para modelizar la enfermedad.



*“Entender el desarrollo embrionario editando el gen *TCOF1* en un modelo animal puede proporcionar información útil para la comprensión de la patología y ensayar posibles tratamientos”.*

Potencial para tratar 6000 desórdenes genéticos... que hay en la clínica hoy? (30 ensayos clínicos)



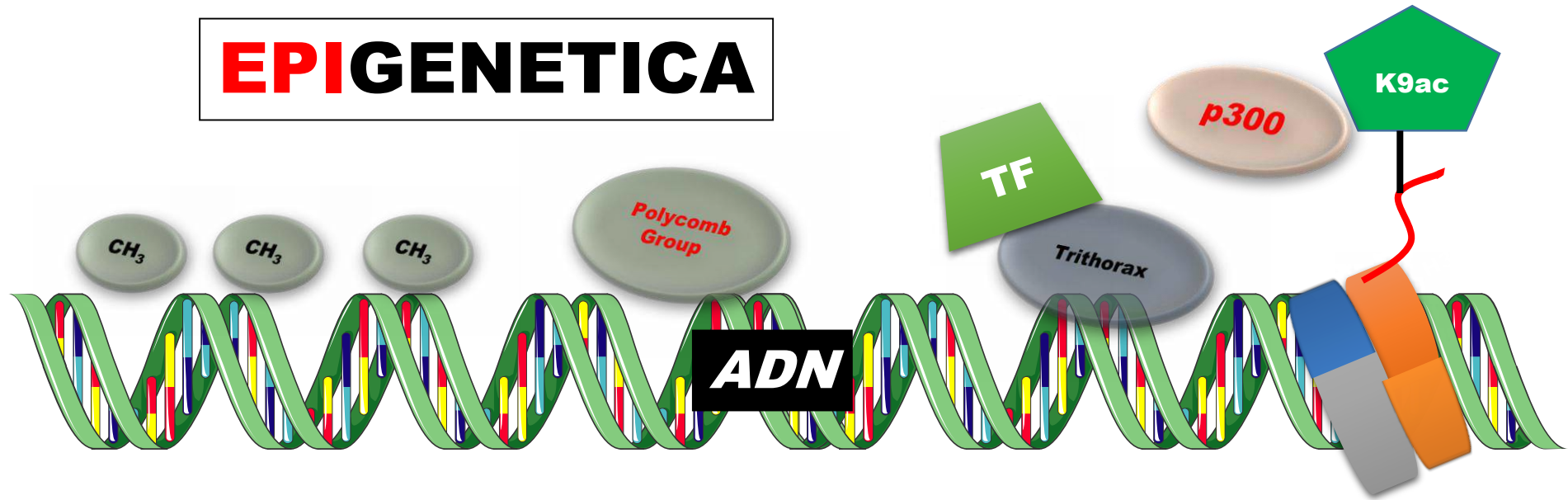
July, Editas and Allergan
(July 2019, Mass, USA).

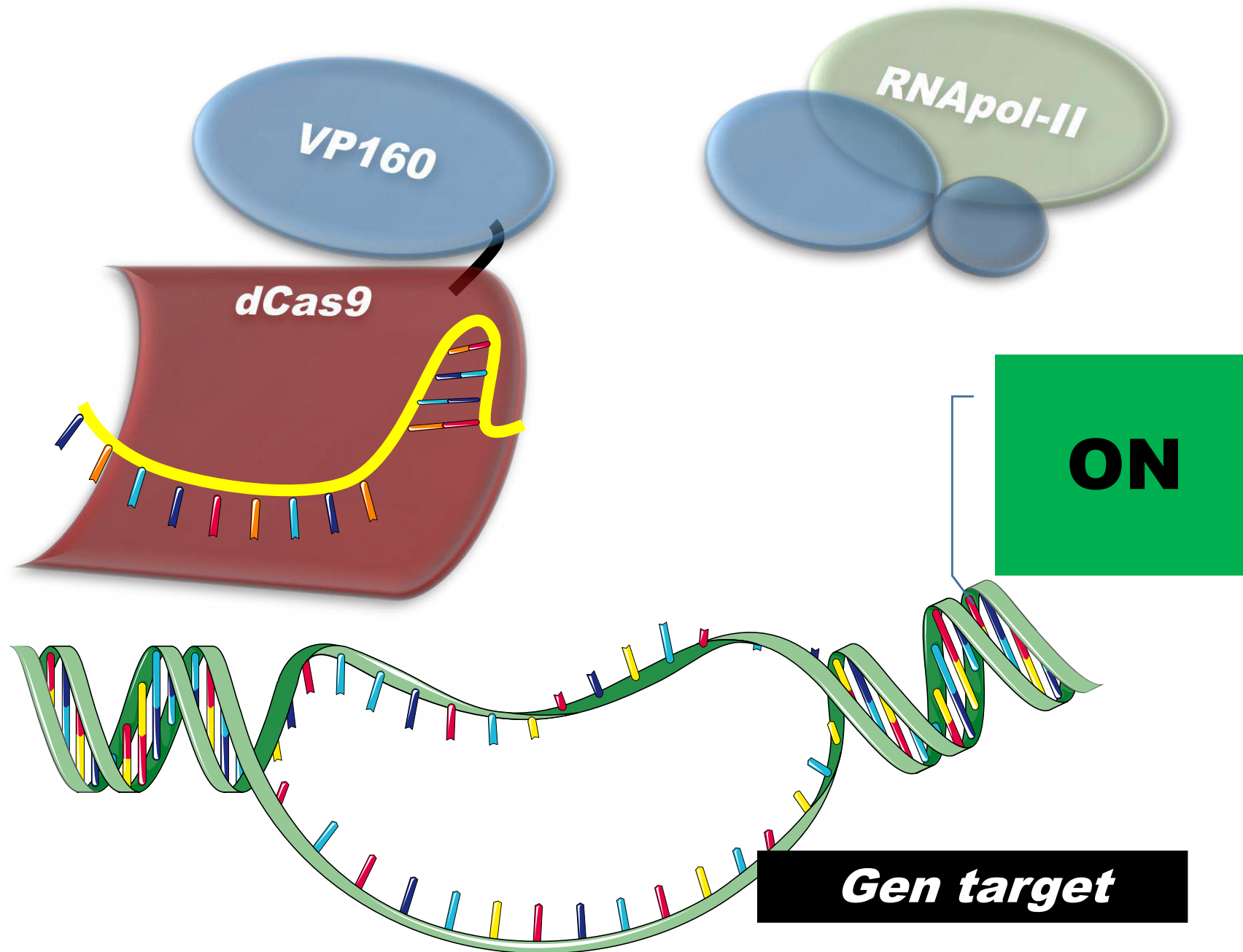
- ❖ Terapia en el cuerpo humano
- ❖ Ceguera hereditaria **Amaurosis congénita de Leber 10**
- ❖ Mutación en el gen CEP290 causa muerte de bastones.
- ❖ Inyección de CRISPR bajo la retina.
- ❖ Regeneración 60% en ratones, 28% en monos

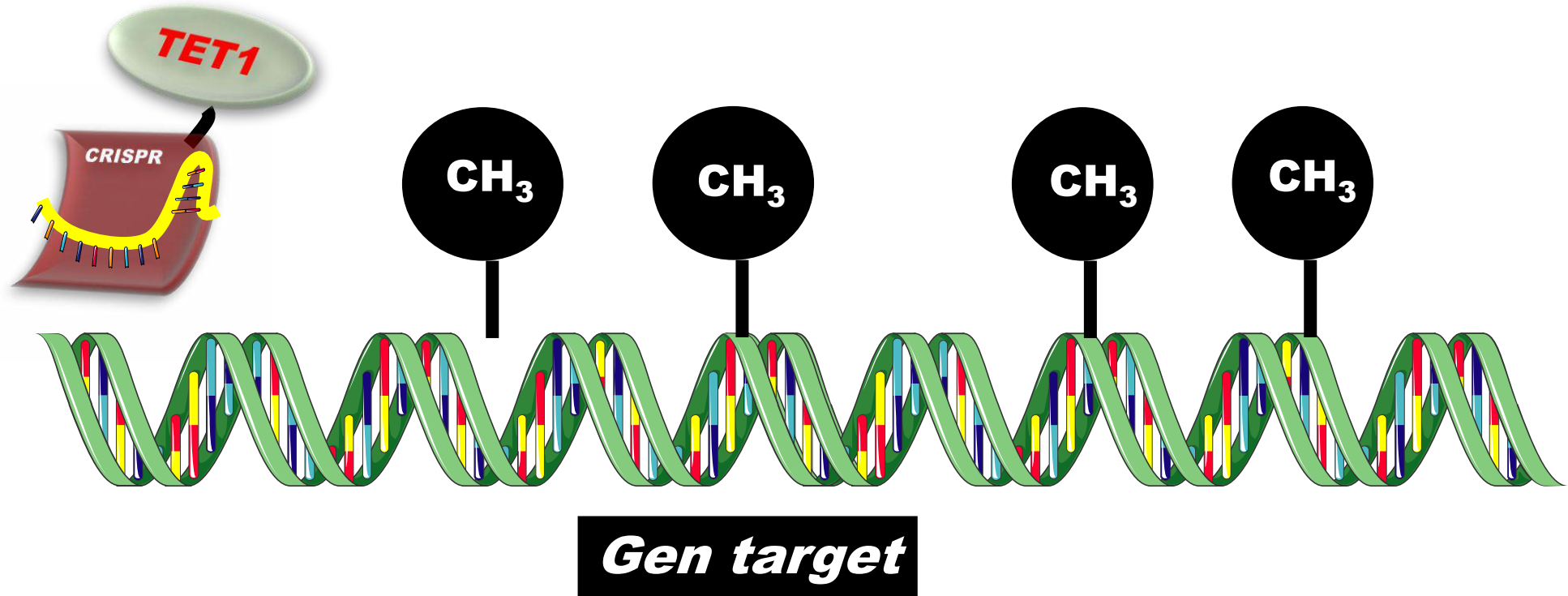


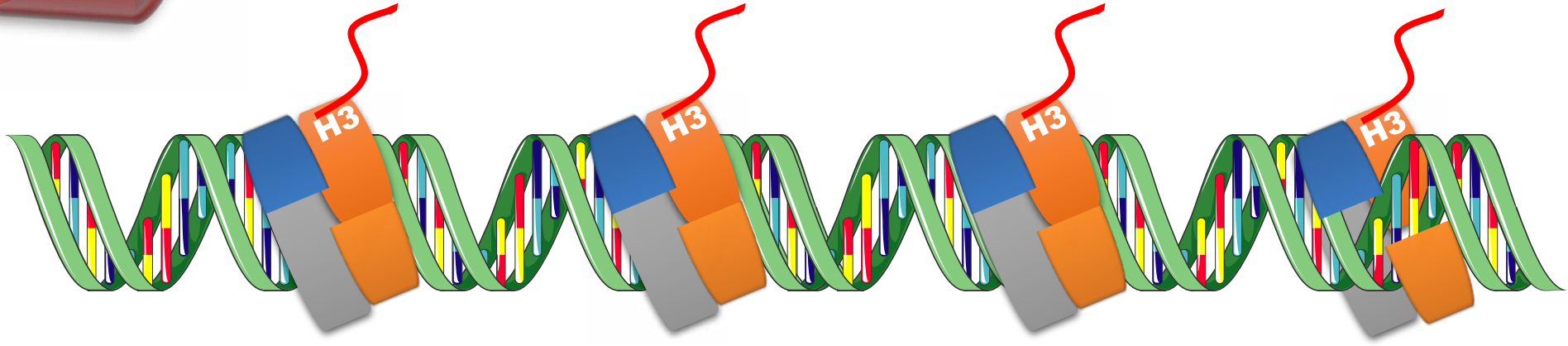
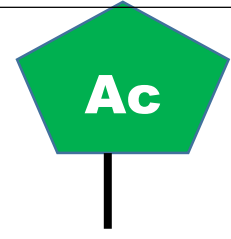
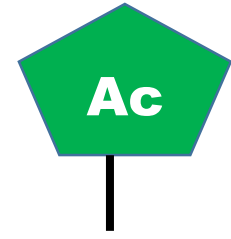
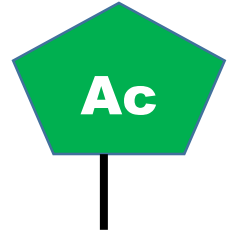
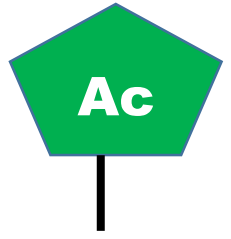
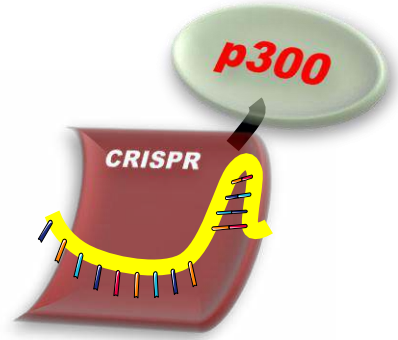
. Edición Epigenética

EPIGENETICA









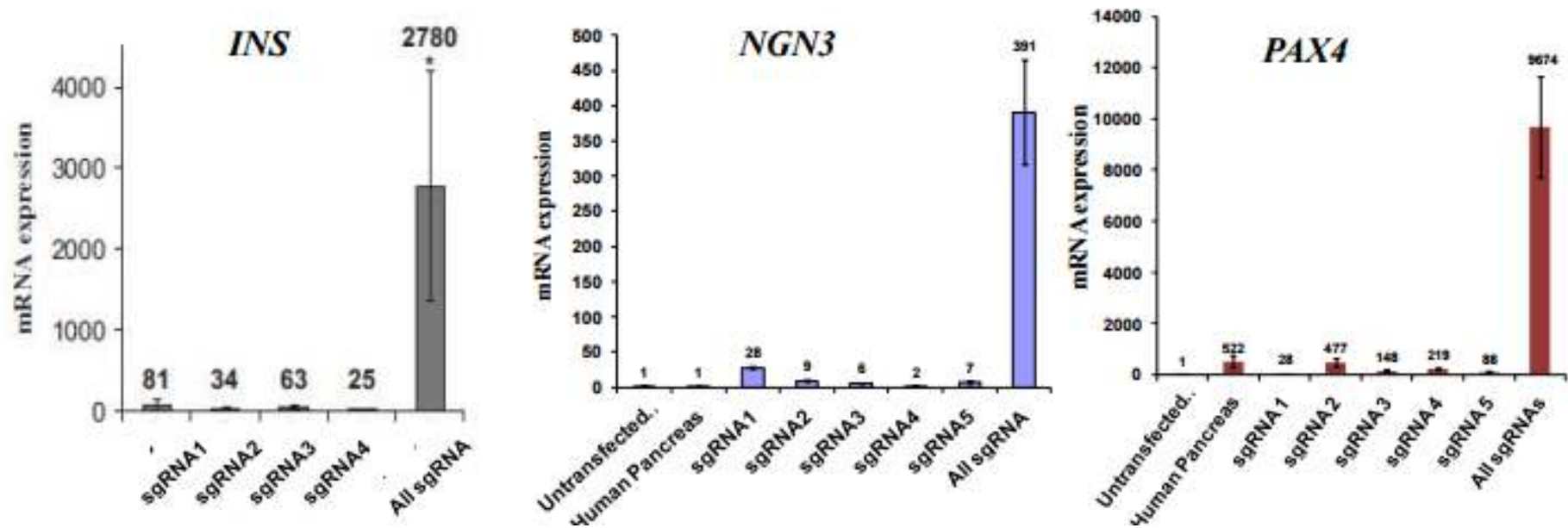
Gen target

Editor's Choice

ORIGINAL ARTICLE
[CRISPR-on system for the activation of the endogenous human INS gene](#) **FREE**

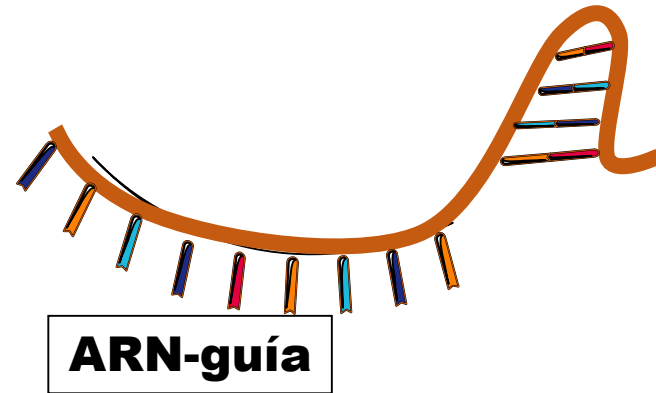
C A Giménez, M Ielpi, A Mutto, L Grosembacher, P Argibay and F Pereyra-Bonne

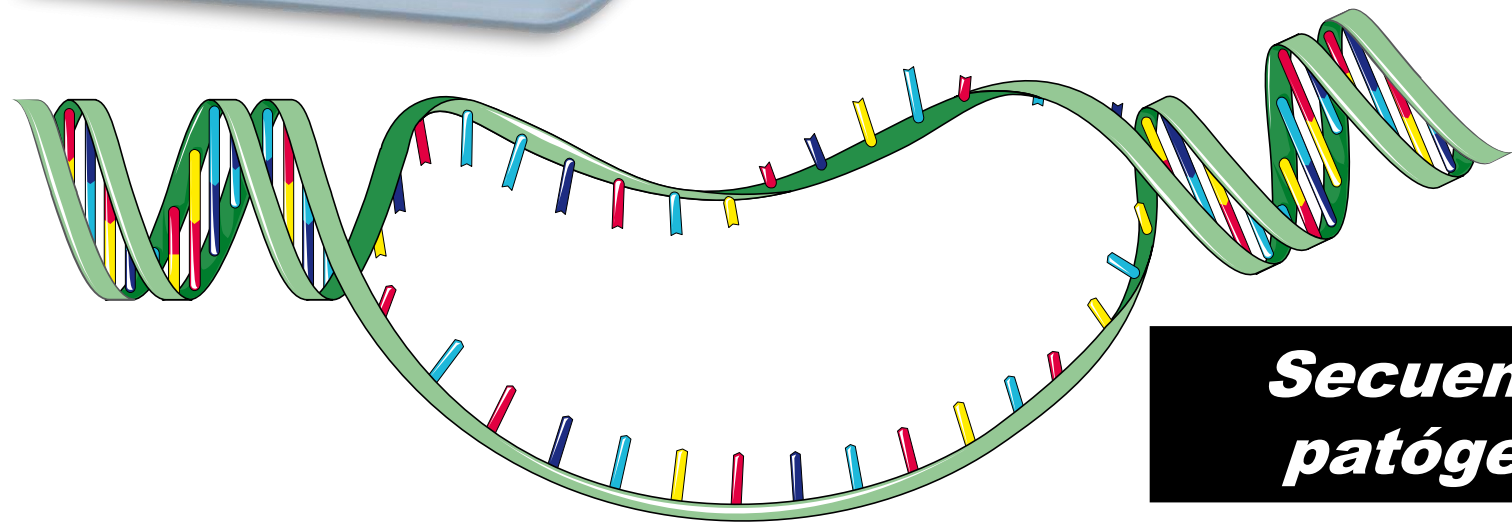
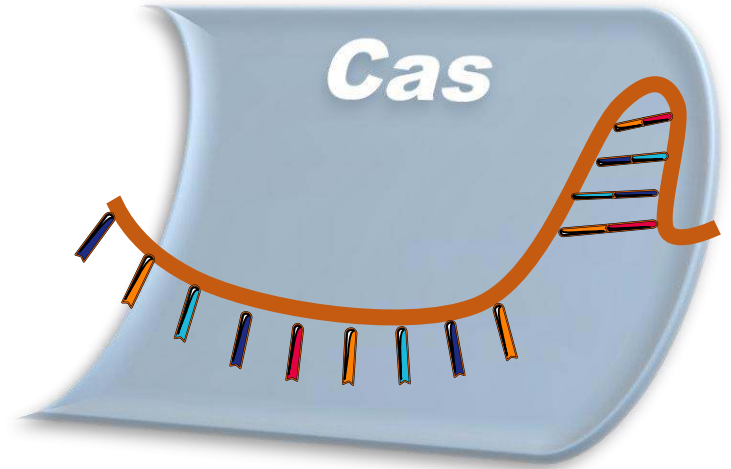
GT advance online publication 7 April 2016; doi: 10.1038/gt.2016.28





. Tratamiento/Diagnóstico





Secuencia patógeno

Existen muchos sistemas CRISPR-Cas

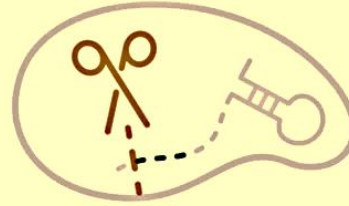
Cas 9



Cas 12



Cas 13



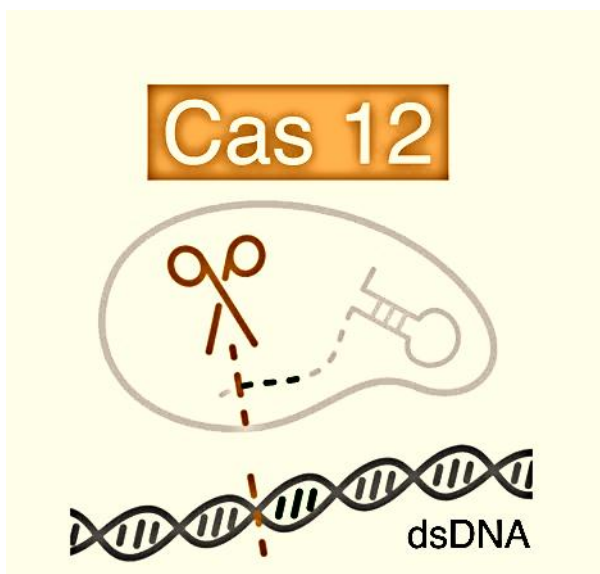
Cas 14



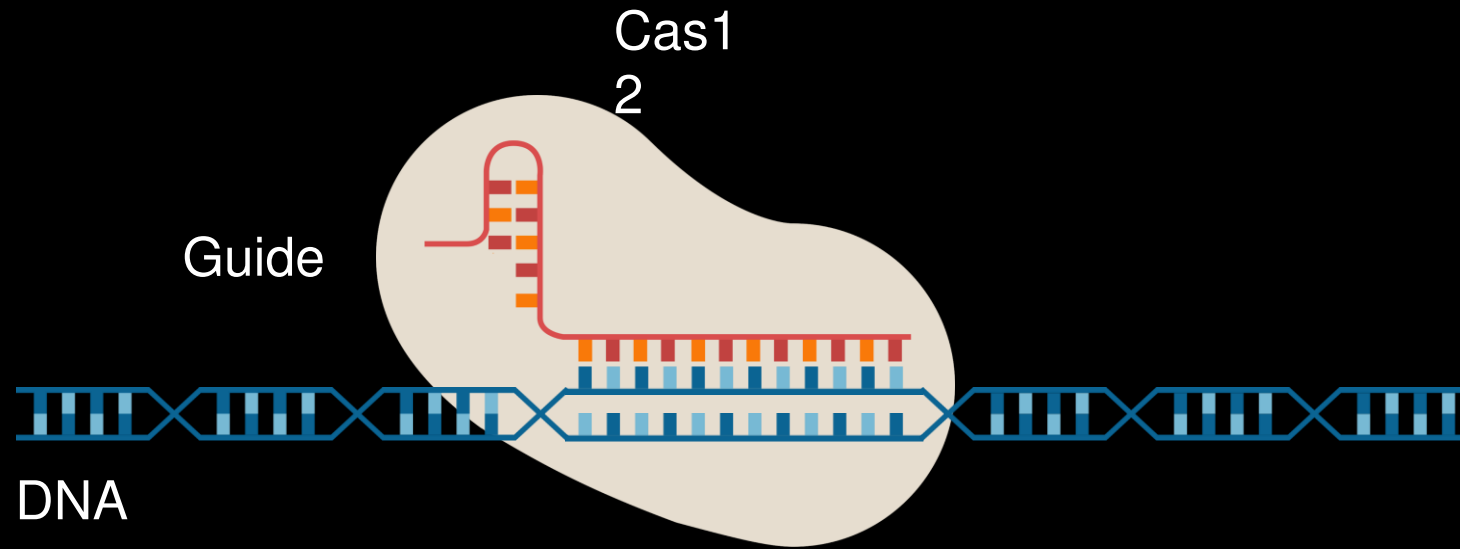
Cite as: J. S. Chen *et al.*, *Science* 10.1126/science.aar6245 (2018).

CRISPR-Cas12a target binding unleashes indiscriminate single-stranded DNase activity

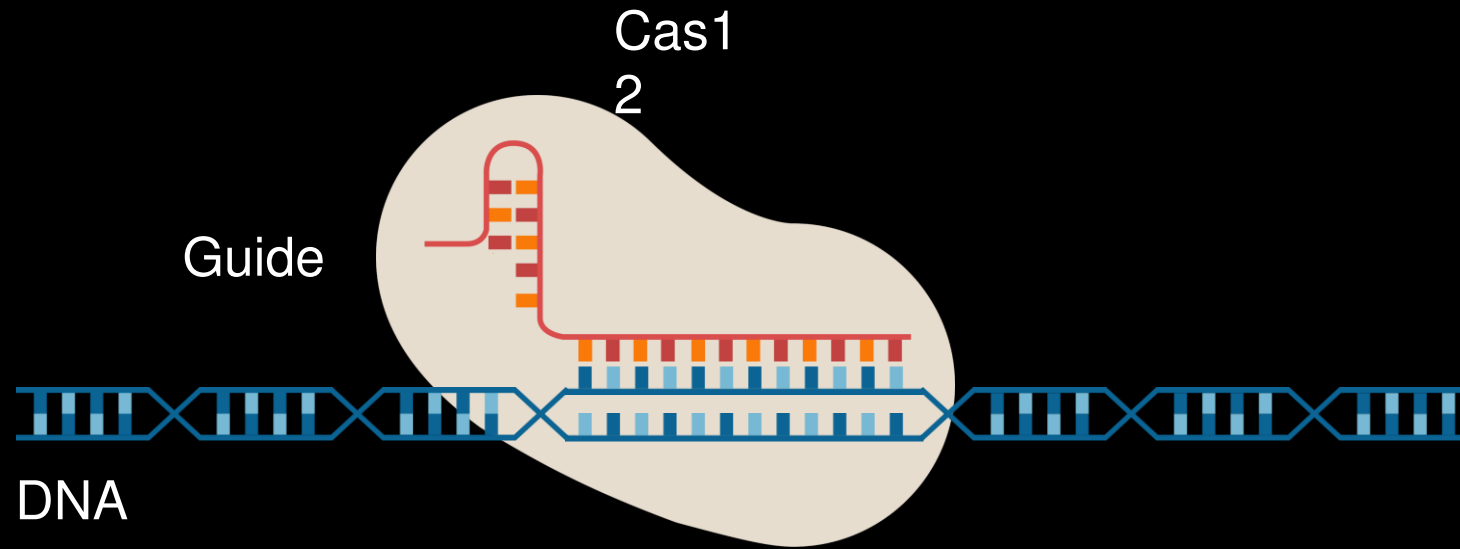
Janice S. Chen,^{1*} Enbo Ma,^{1*} Lucas B. Harrington,^{1*} Maria Da Costa,² Xinran Tian,³ Joel M. Palefsky,² Jennifer A. Doudna^{1,3,4,5,6†}



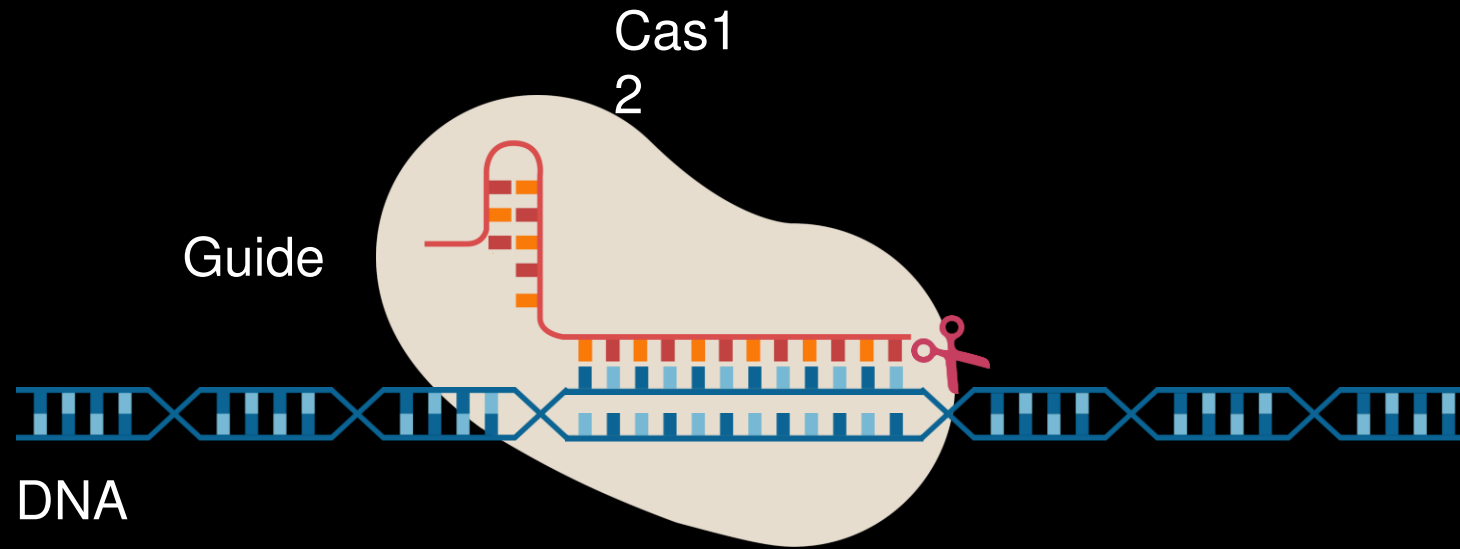
CRISPR Diagnostics



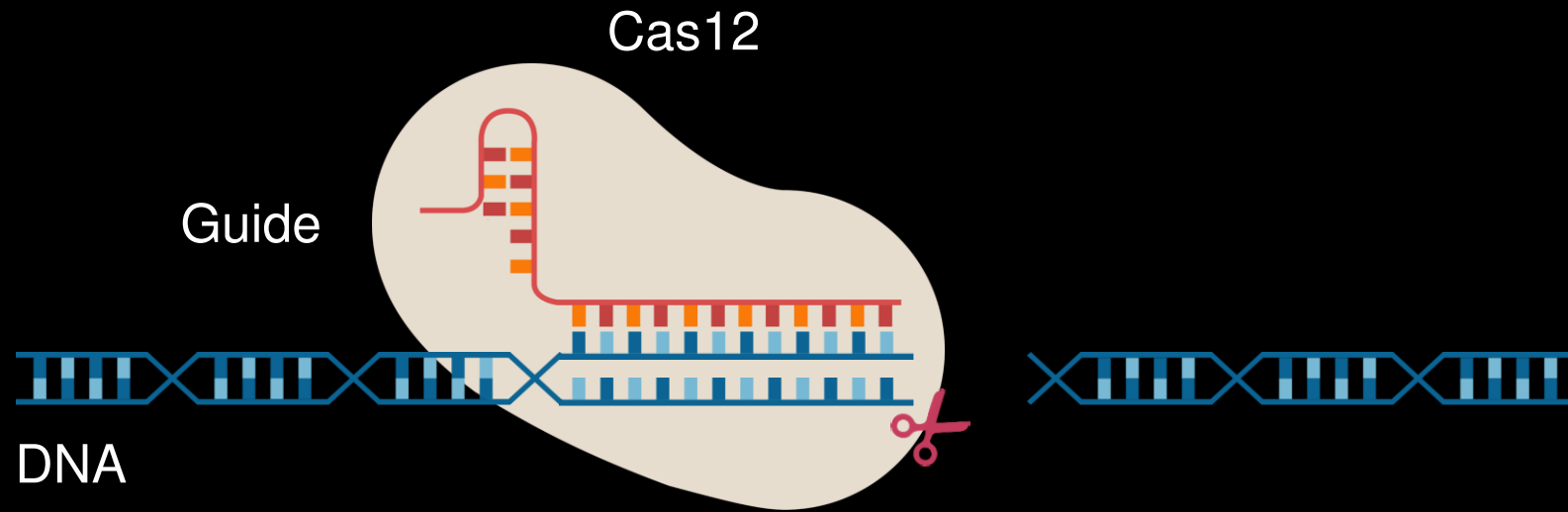
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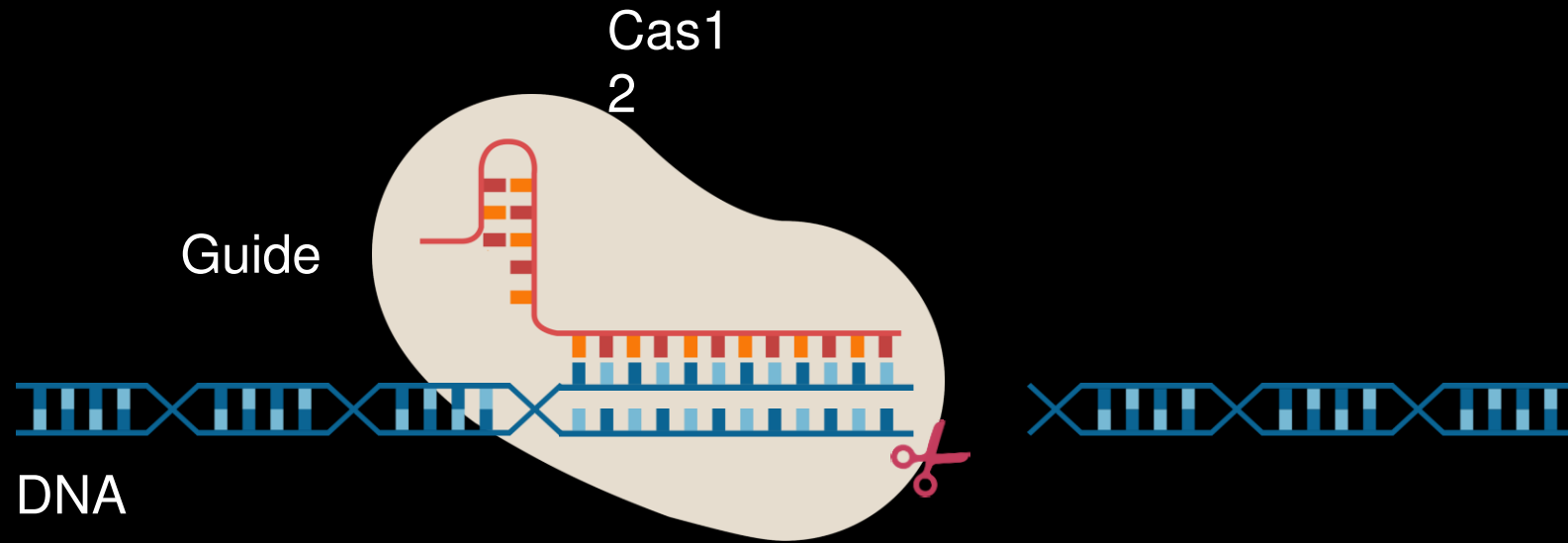
CRISPR Diagnostics



CRISPR Diagnostics

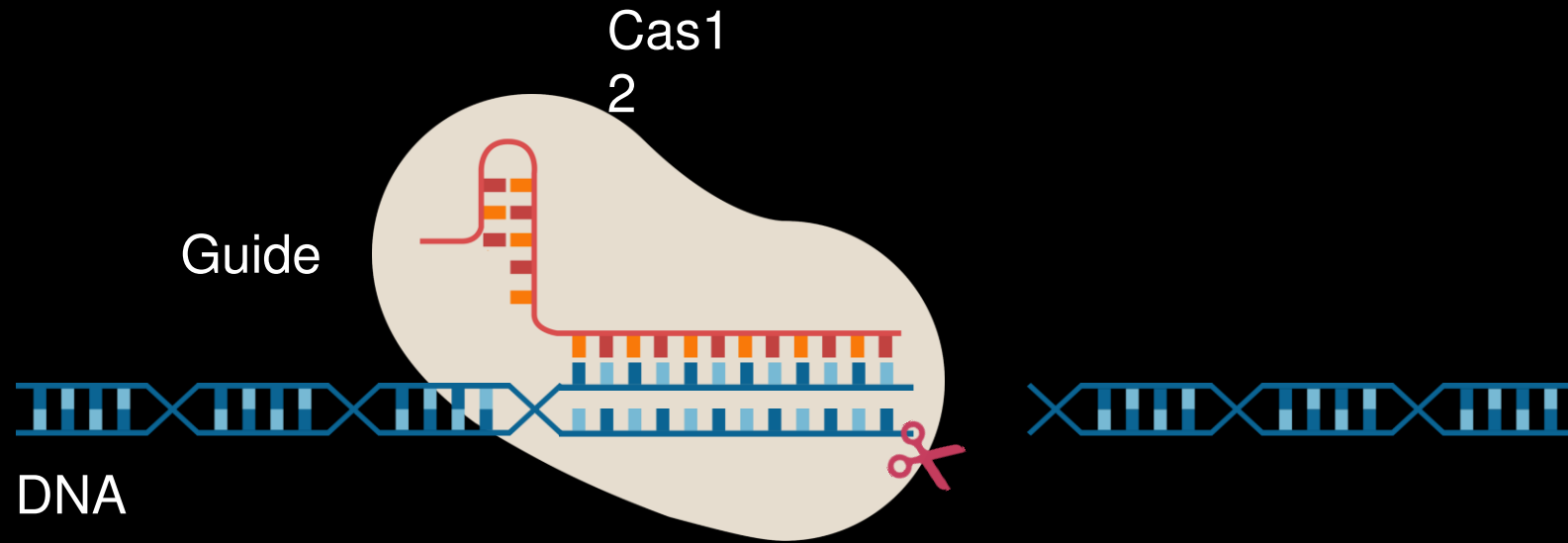


CRISPR Diagnostics



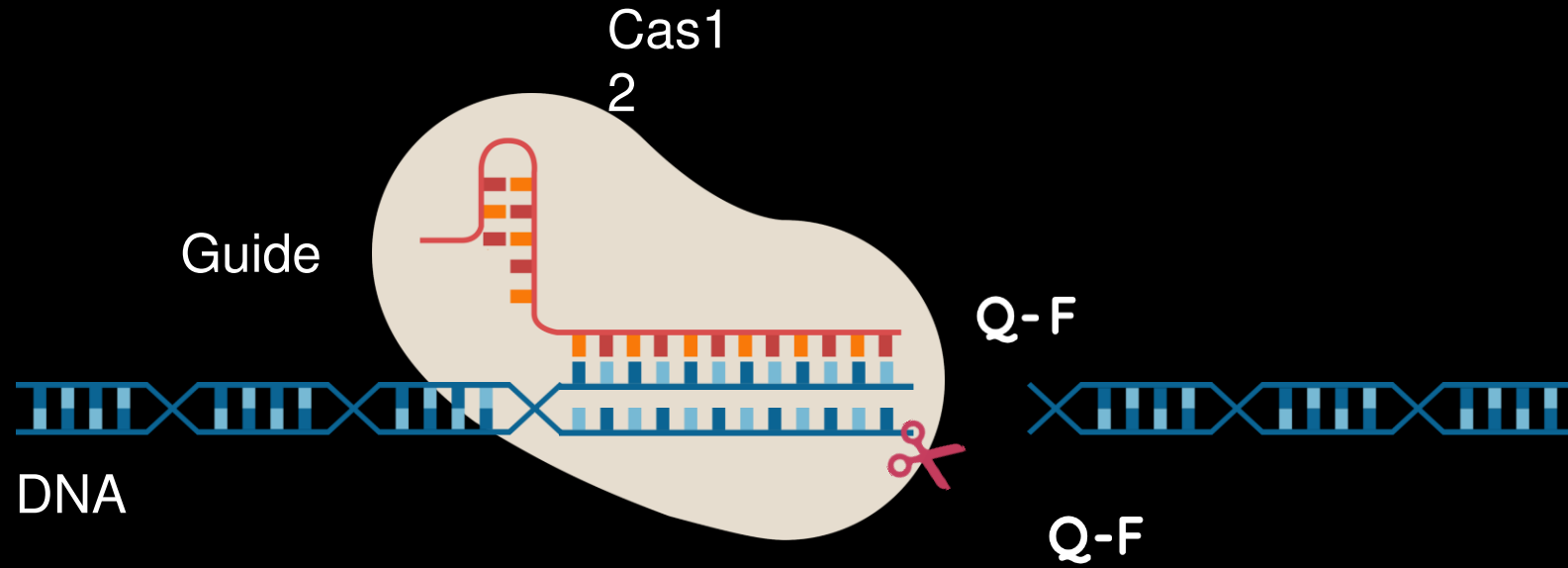
Actividad Colateral

CRISPR Diagnostics



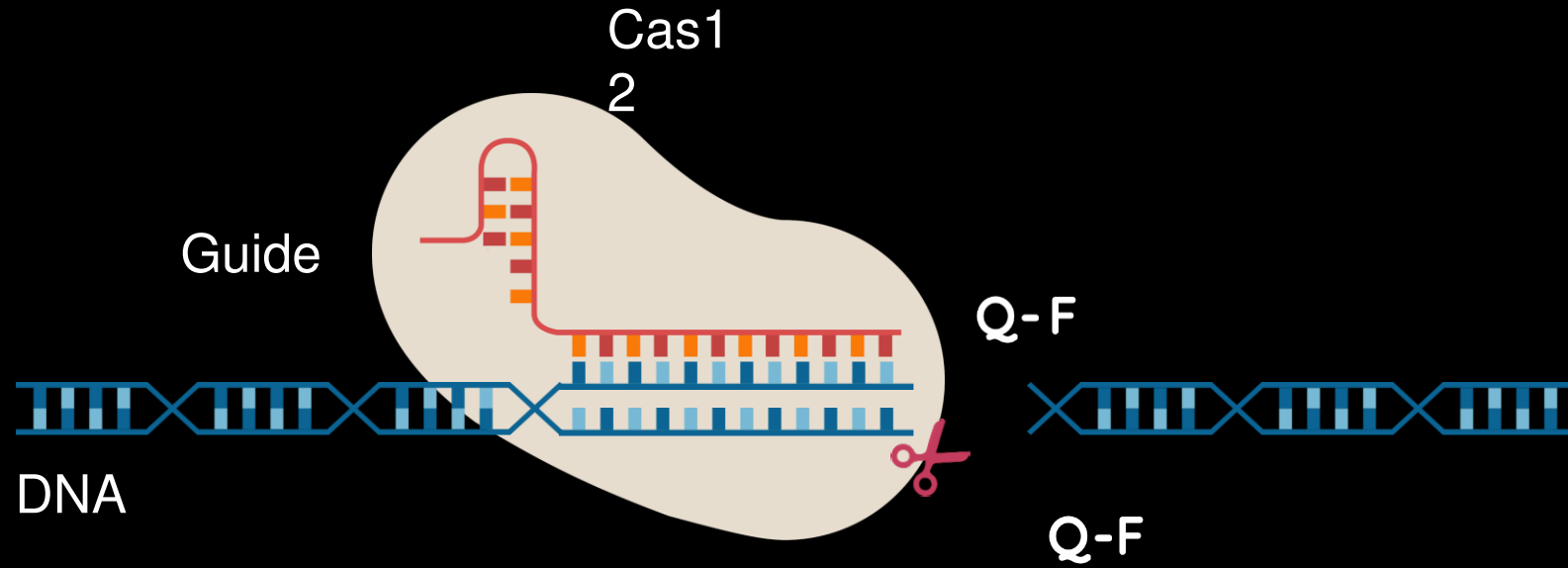
Actividad Colateral

CRISPR Diagnostics



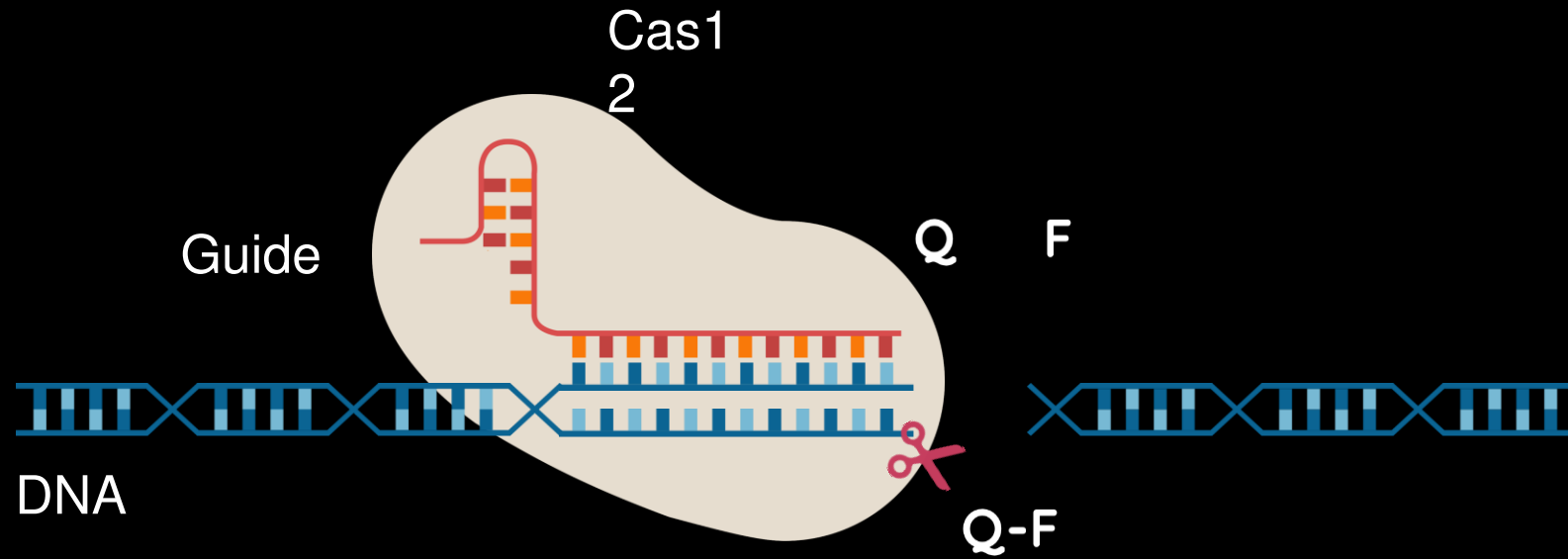
Actividad Colateral

CRISPR Diagnostics



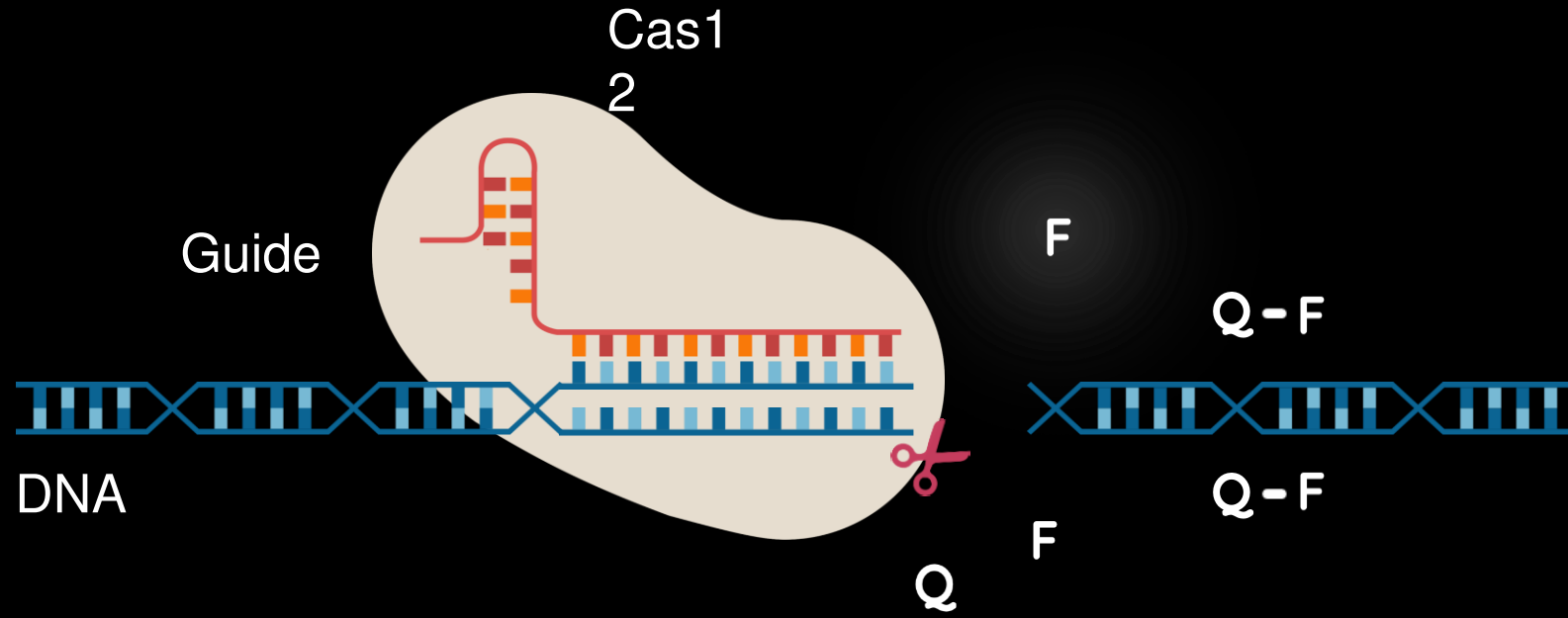
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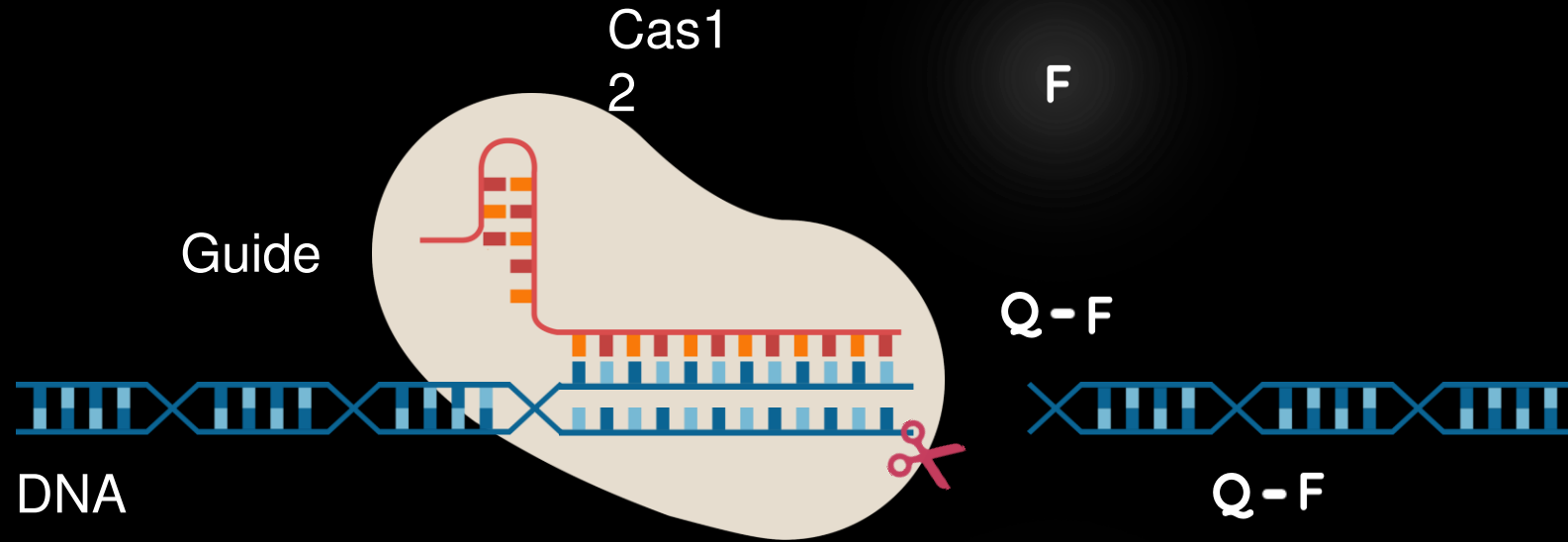
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CRISPR Diagnostics



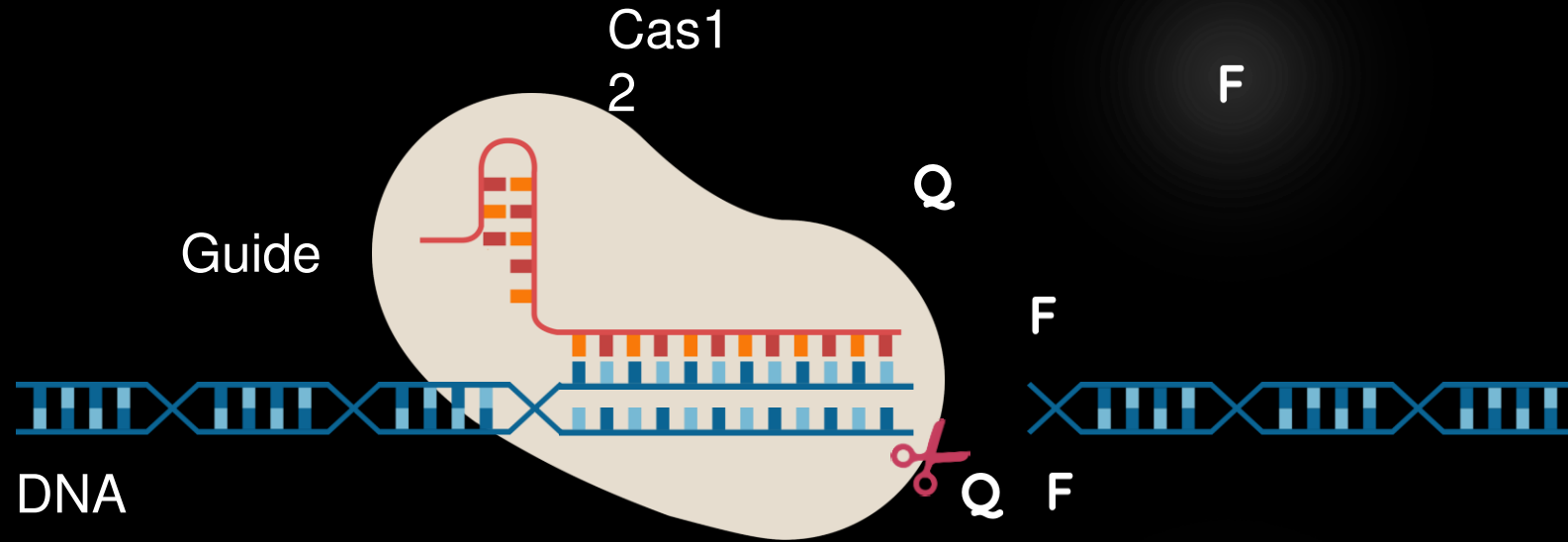
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CRISPR Diagnostics



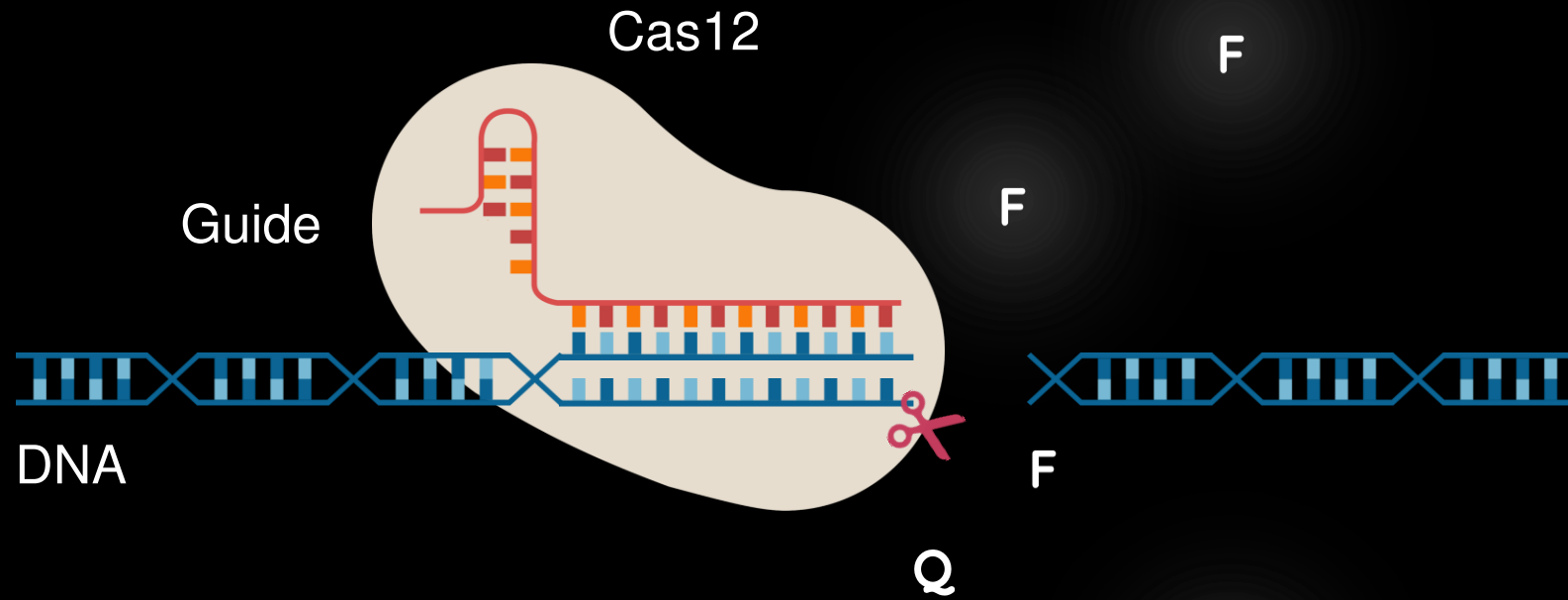
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CRISPR Diagnostics



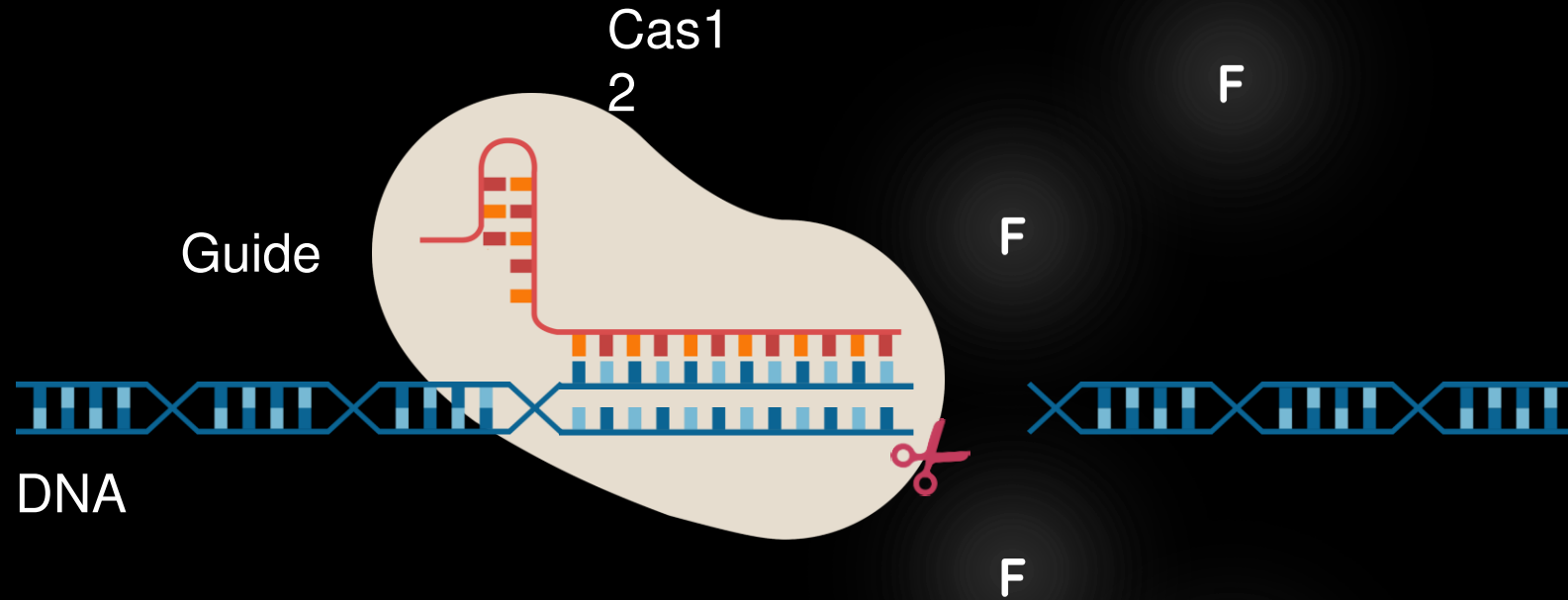
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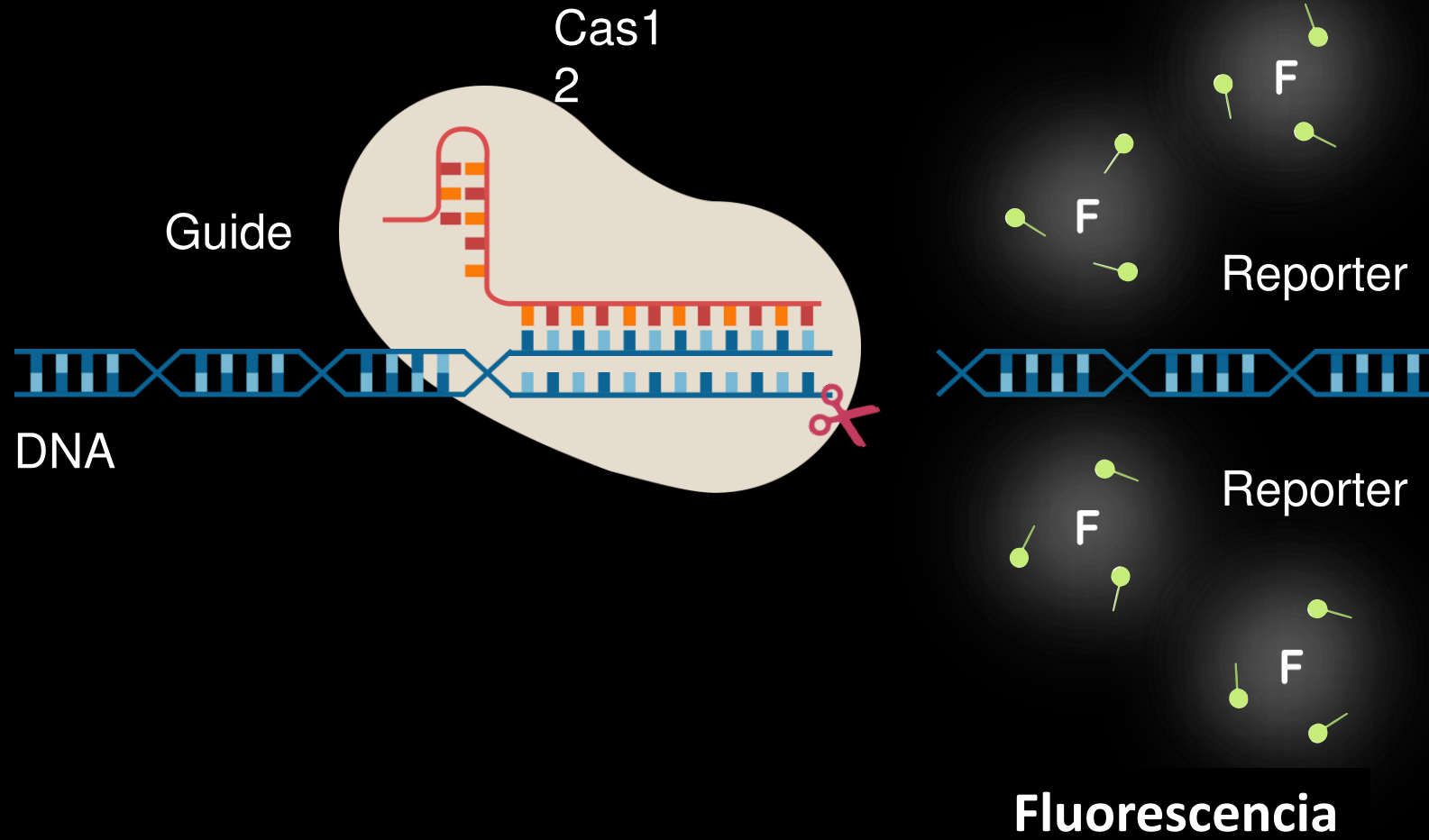
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CRISPR Diagnostics

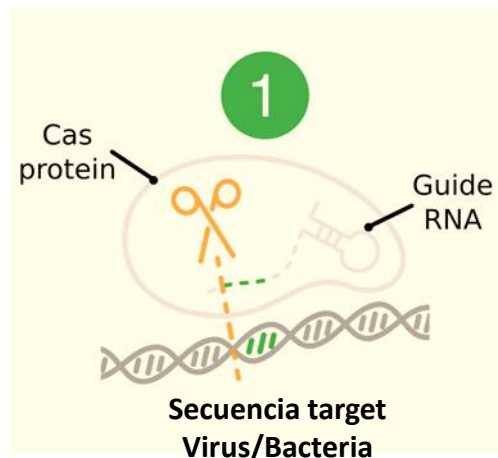


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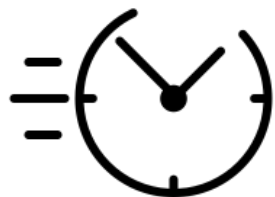
CRISPR Diagnostics



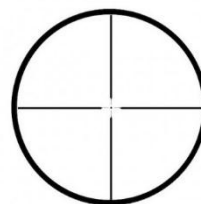
CRISPR-Cas12 Diagnóstico



Ventajas del sistema de detección



Poca purificación



A microscopic view of numerous purple, spherical bacteria, likely cocci, arranged in various chains and clusters against a black background. The bacteria have a textured, granular surface.

BACTERIAS SUPER RESISTENTES

Existe la necesidad de identificar
la naturaleza de la resistencia
para un eficiente tratamiento.

Detección de resistencias

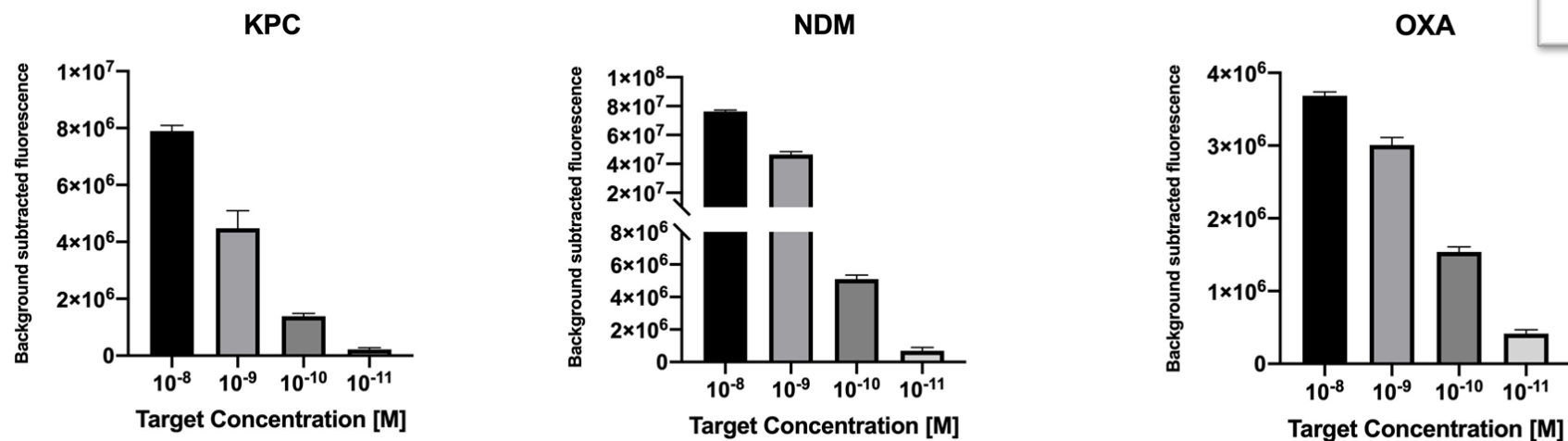


Figura 4. Detección de secuencias sintéticas de KPC, NDM y OXA por CRISPR. Valores a los 30 min en un lector de placas (TecanF2000) en 40 uL (límite de detección 10 picomolar).

Curti et al., unpublished data.



DENGUE
ZIKA
HANTAVIRUS



Detección de virus

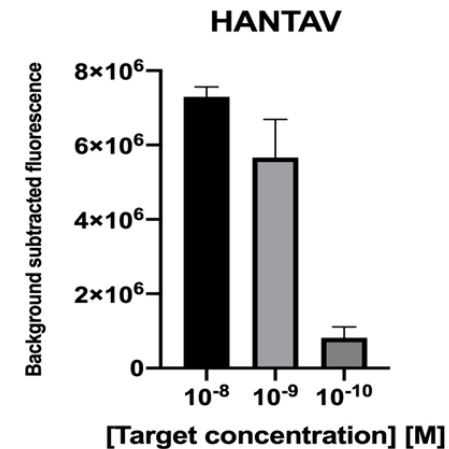
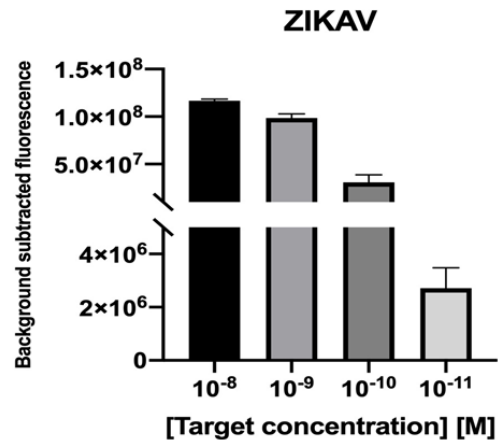
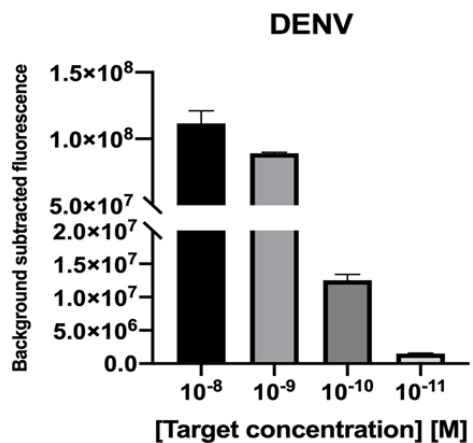


Figura 1. Detección de secuencias sintéticas de dengue, zika y hantavirus por CRISPR. Valores a los 30 min en un lector de placas (TecanF2000) en 40 uL. En todos los casos se incluyó un paso inicial de RT (detección picomolar).

Curti et al., unpublsh data.



CAASPR

BIOTECH

Next generation of CRISPR diagnostics

(startup bajo normativa CONICET)

2019



El Equipo



Franco Goytia

CEO - B.Sc. Economics
Healthcare Specialization



Carla Giménez

CSO - PhD Candidate CRISPR



Federico Pereyra-Bonnet

CoFounder - BSc, PhD CRISPR
(INPA-UBA-CONICET)



Lucia Curti, BSc

Synthetic RNAs

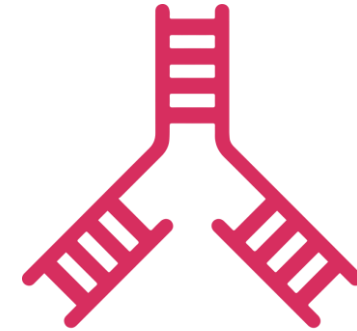


Guillermo "Coco" Repizo

Bioinformatics - BSc PhD Microbiology
(IBR-CONICET)

Escenario

GRIDX

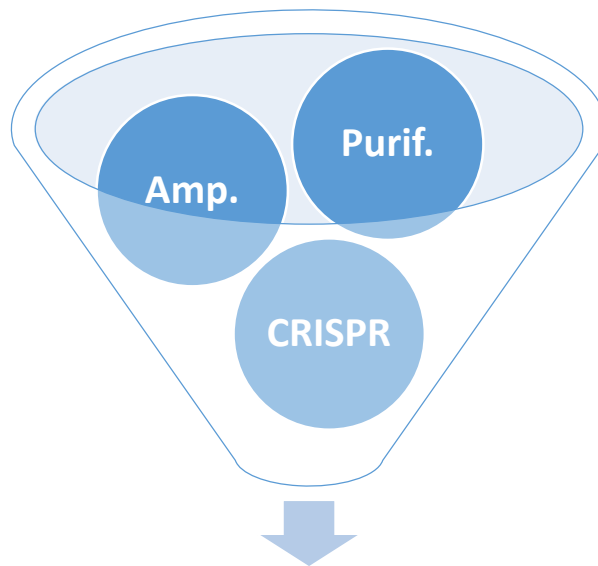


INDIE BIO
ACCELERATING BIOLOGY

CONICET



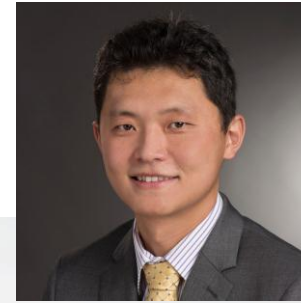
Desafíos de CASPR



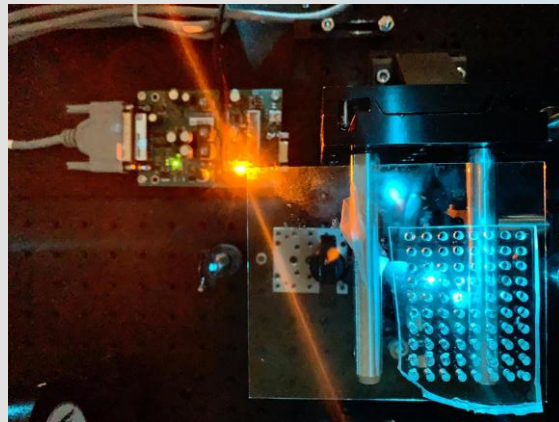
AUTOMATIZACIÓN

PORTABILIDAD

DIGITALIZACIÓN



We already have developed a CRISPR + Microfluidics Prototype



Results in < 30 minutes

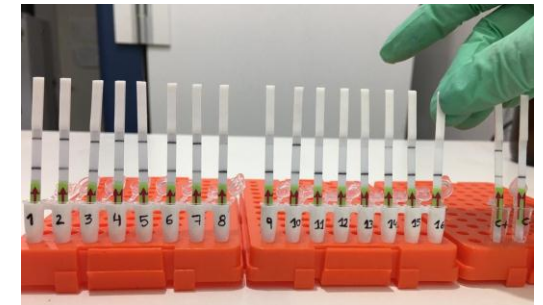
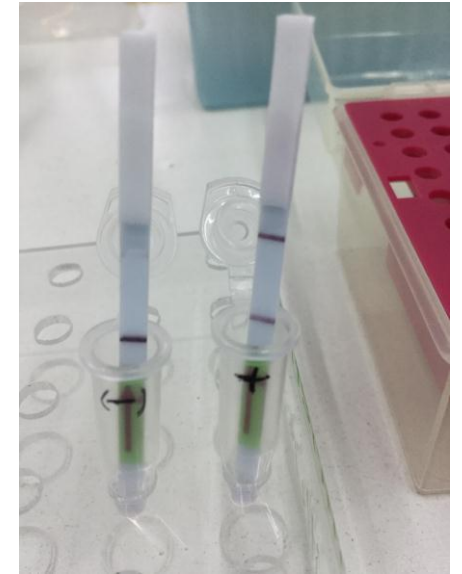
Minimal sample preparation

Attomolar Sensitivity



PORTABILIDAD

- Test portable para Dengue
- Sin equipamiento
- USD 0,6/2 muestra (costo)
- >60 min
- Muestras humanas



CONICET



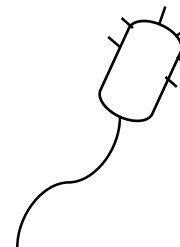
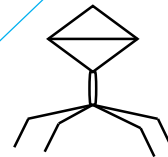
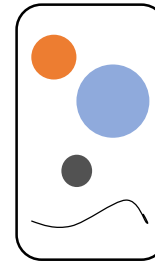
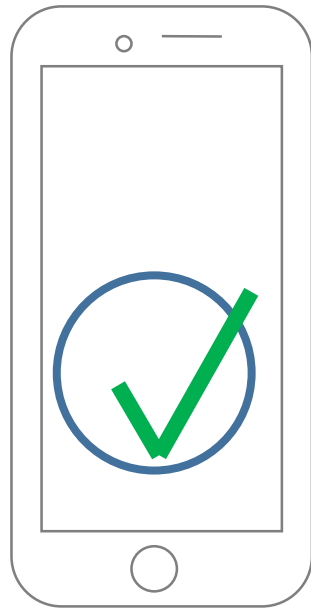
U Na M

INSTITUTO DE BIOLOGÍA SUBTROPICAL

Dr Marcos Miretti
Protocolo y CI aprobado

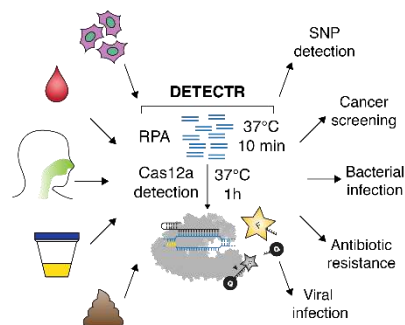


Tarjeta reactiva compatible con cualquier teléfono smart



GAATGGC
CTTACCG

POTENCIAL IMPACTO



The image features a diver in the upper left corner, spearing a fish in a deep blue underwater environment. Sunlight rays filter through the water. In the foreground, a large, open oyster shell rests on a rock, with a single, large, white pearl centered inside it. The text "The CasPR Opportunity" is overlaid in the center, with "Cas" in blue and "PR Opportunity" in white.

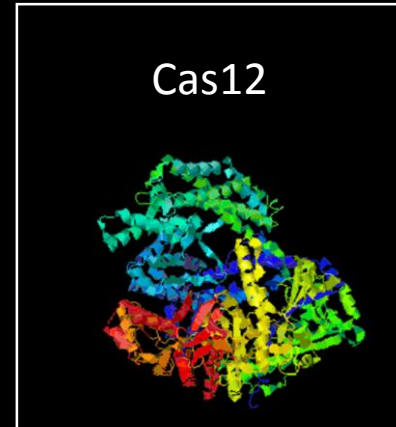
“The CasPR Opportunity”

1. Golfo Nuevo, Patagonia
2. Volcán Lanín, Neuquén
3. Glaciar Perito Moreno, Santa Cruz
4. Marambio, Antartica





PCR



CRISPR
diagnóstico

Muchas
Gracias

