

The background of the slide features a repeating pattern of interlocking puzzle pieces in a light gray color. The pieces are arranged in a grid-like fashion, creating a textured, geometric background.

The human model: A fresh perspective on autism research

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AUTISM

**1 in 54
boys**





US\$ 3.2 MI



GENETICS



ENVIRONMENT

NO CURE



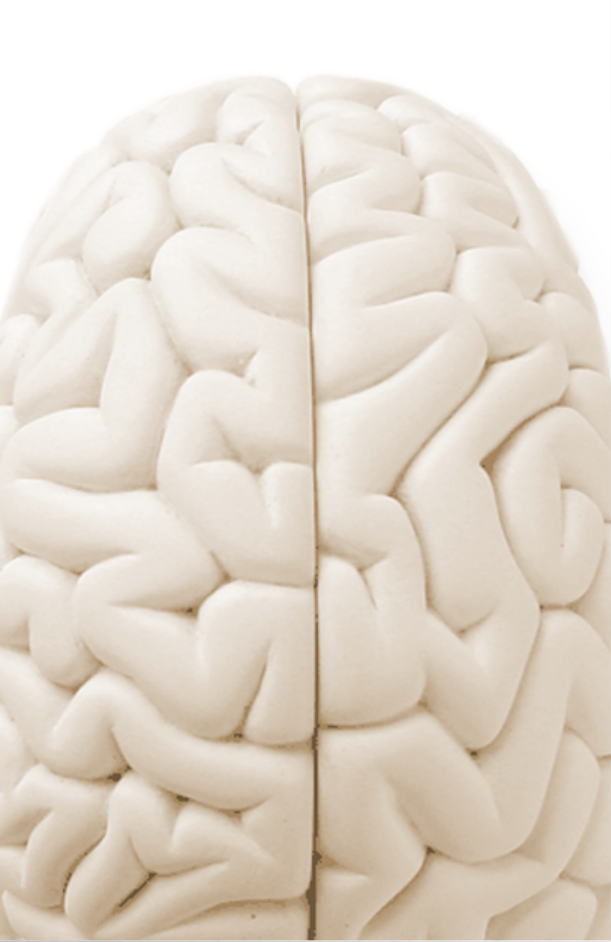


Gisele Bündchen



What is a model?





Postmortem

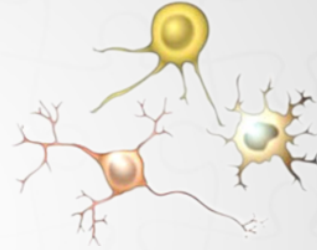


BLOOD

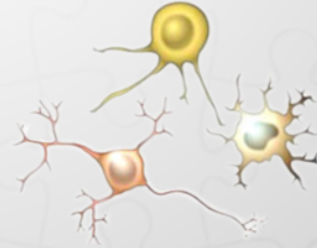


Animals

A new autism model?



Differences?

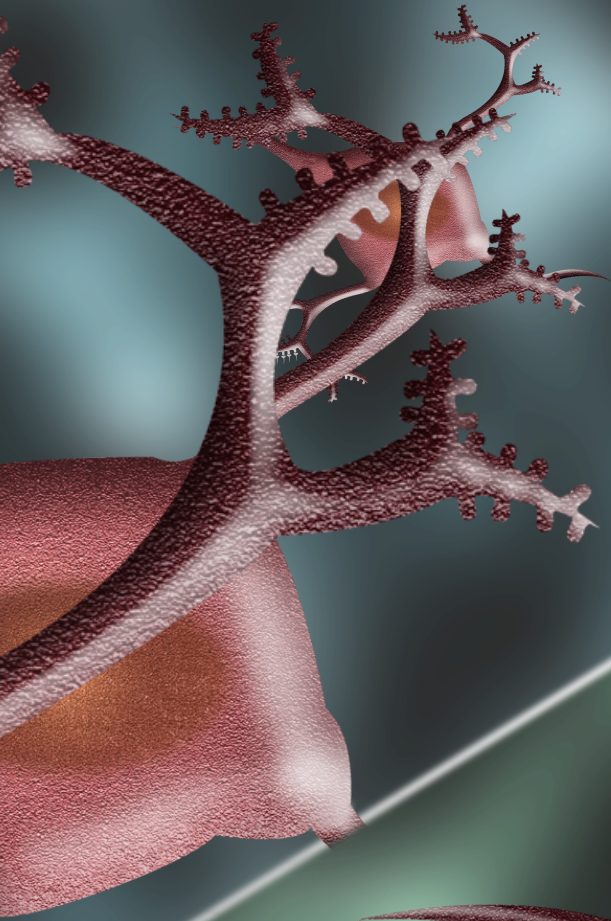




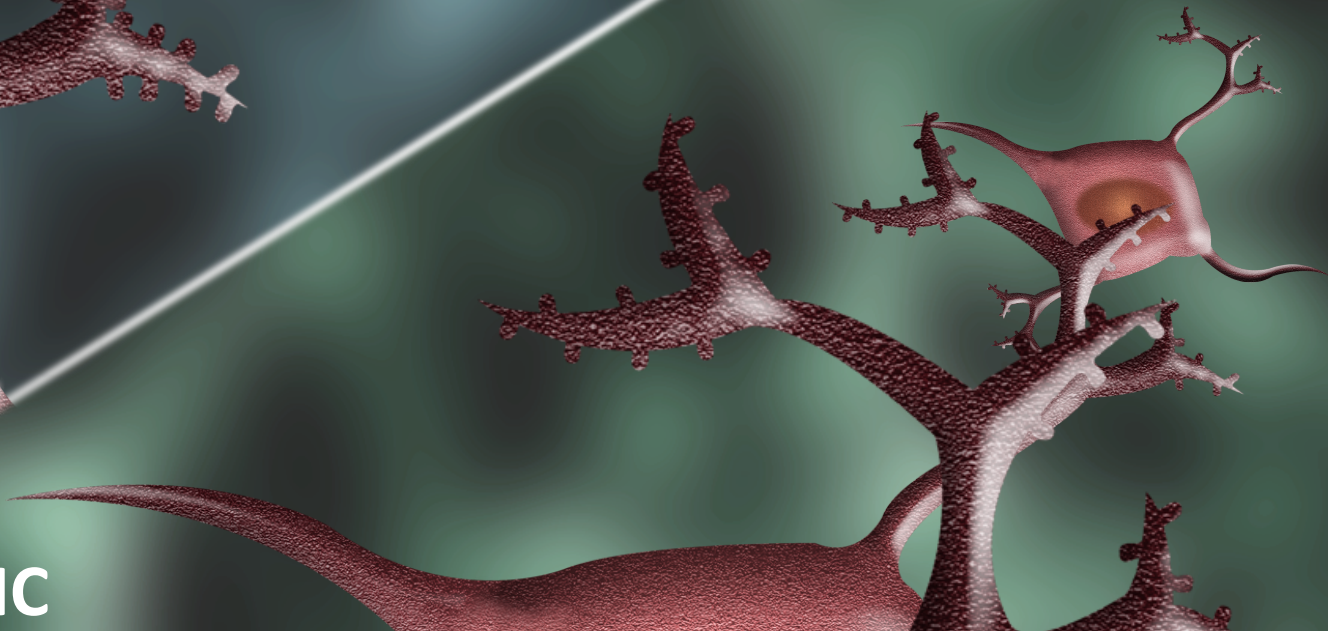
The Tooth Fairy Kit Collection



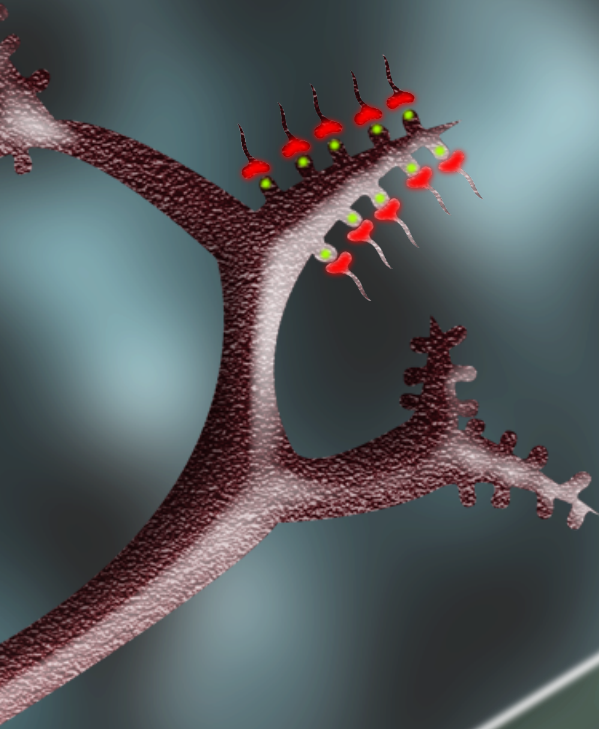
CONTROL



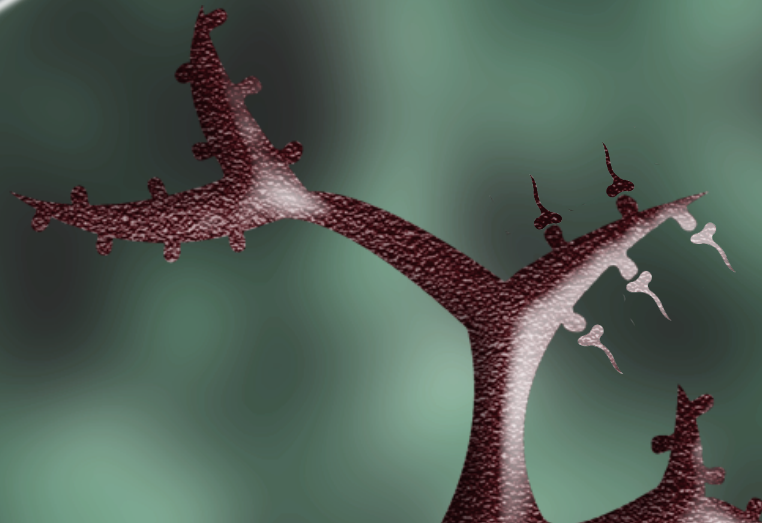
AUTISTIC



CONTROL



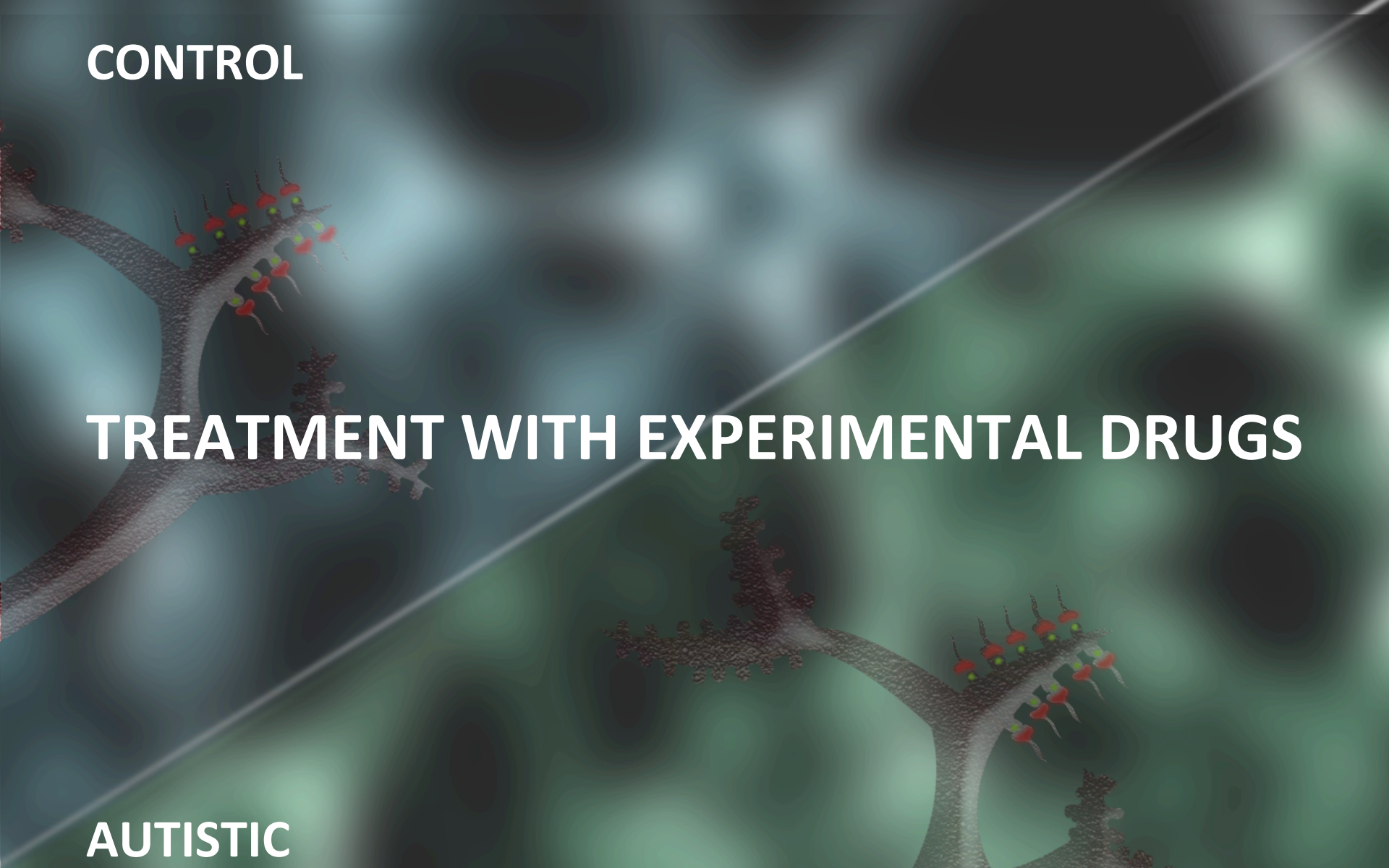
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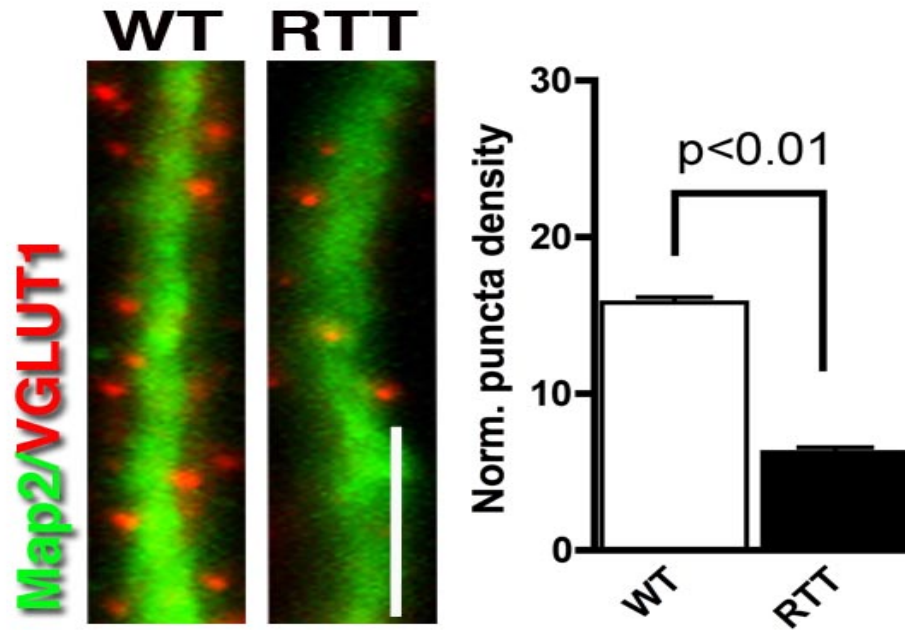
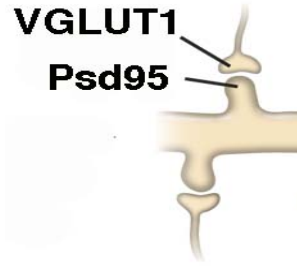
CONTROL

TREATMENT WITH EXPERIMENTAL DRUGS

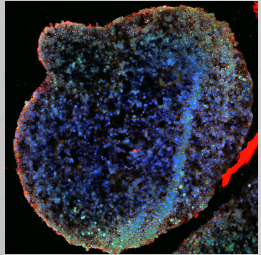
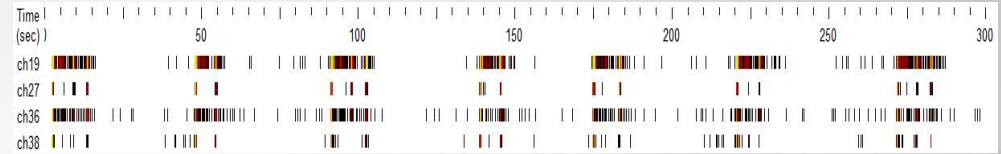
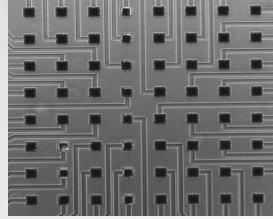
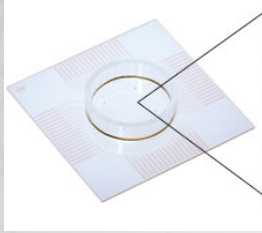
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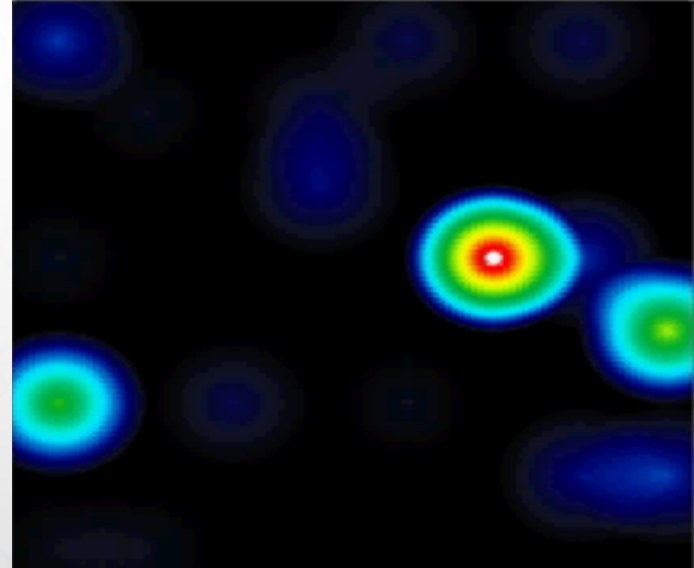
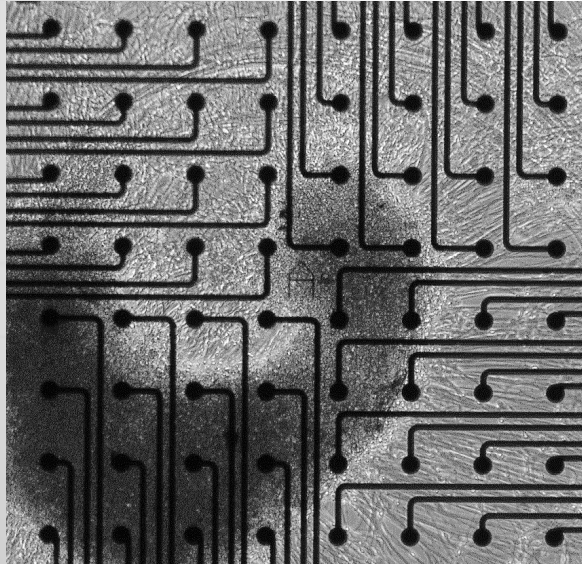
RTT neurons have fewer glutamatergic synapses



Brain on a chip

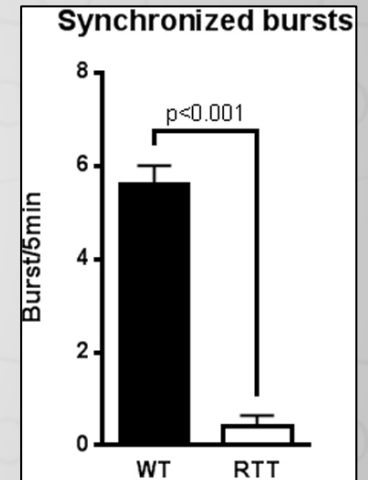
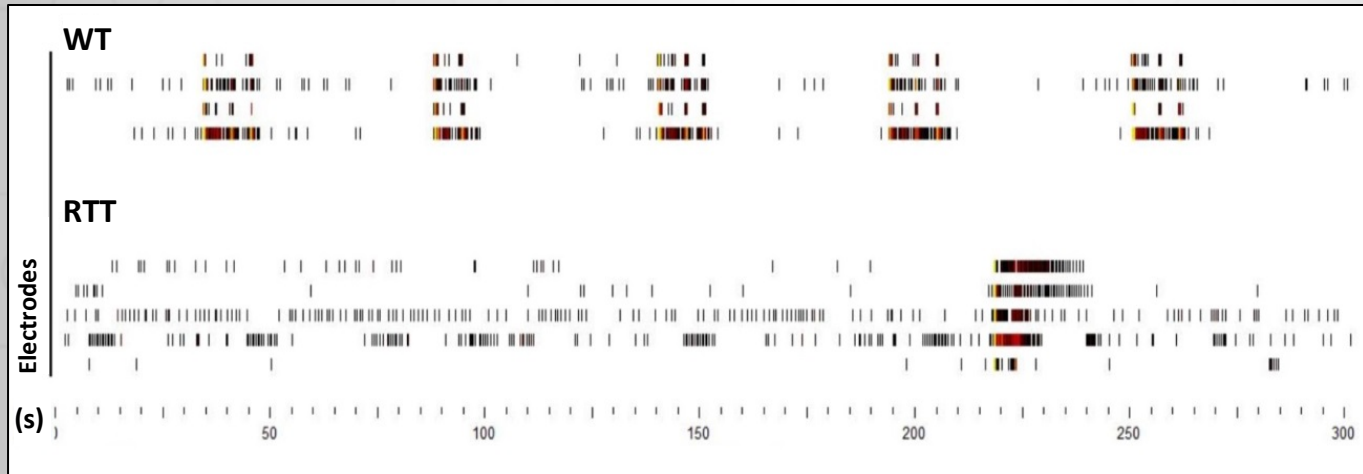


3D corticoids



Activity Map

RTT neuronal networks are not synchronized

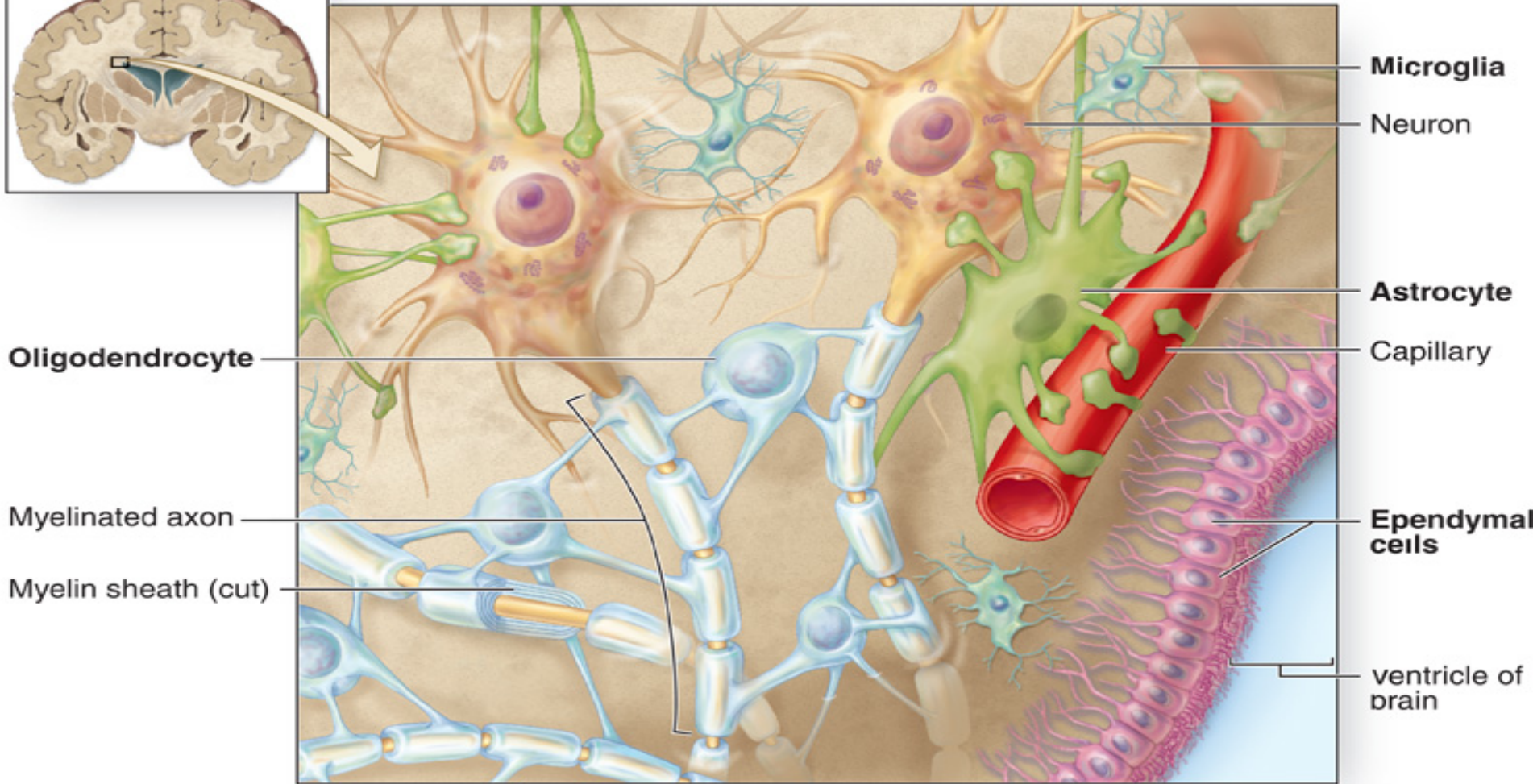
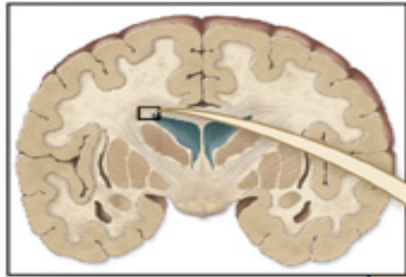


Intractable seizures in a dish

CDKL5 Syndrome:

- Genetically defined by alterations in the *CDKL5* gene
- Early onset of multiple seizures episodes
- Intractable epilepsy
- Two mouse models (deletion/mutation) with no seizures
- Potential benefit from CBD

GLIA



Oligodendrocyte

Myelinated axon

Myelin sheath (cut)

Microglia

Neuron

Astrocyte

Capillary

Ependymal cells

ventricle of brain

ASD astrocytes have impaired communication

WT

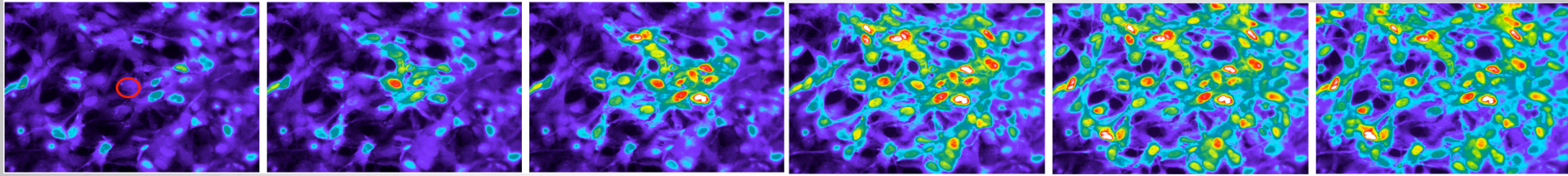
1.5s

1.5s

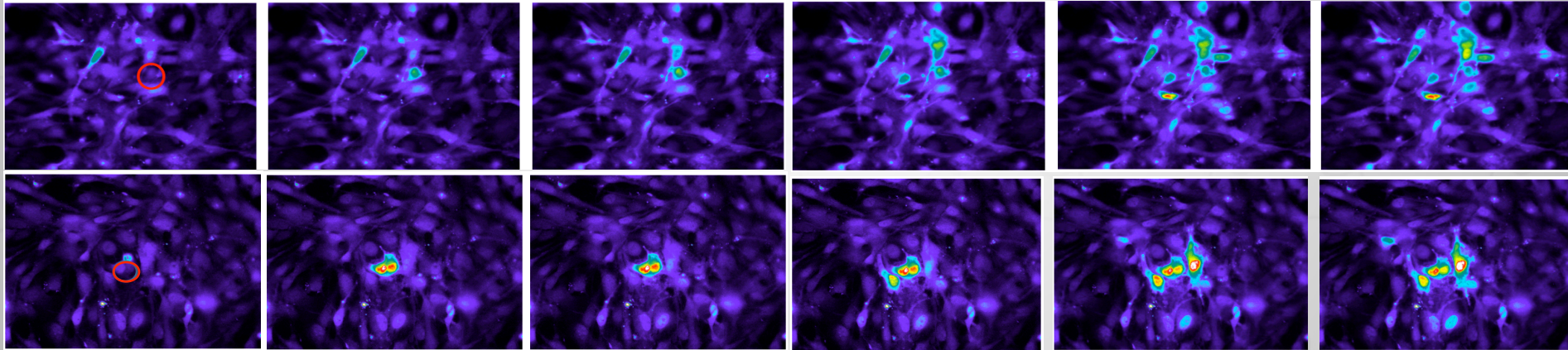
3s

3s

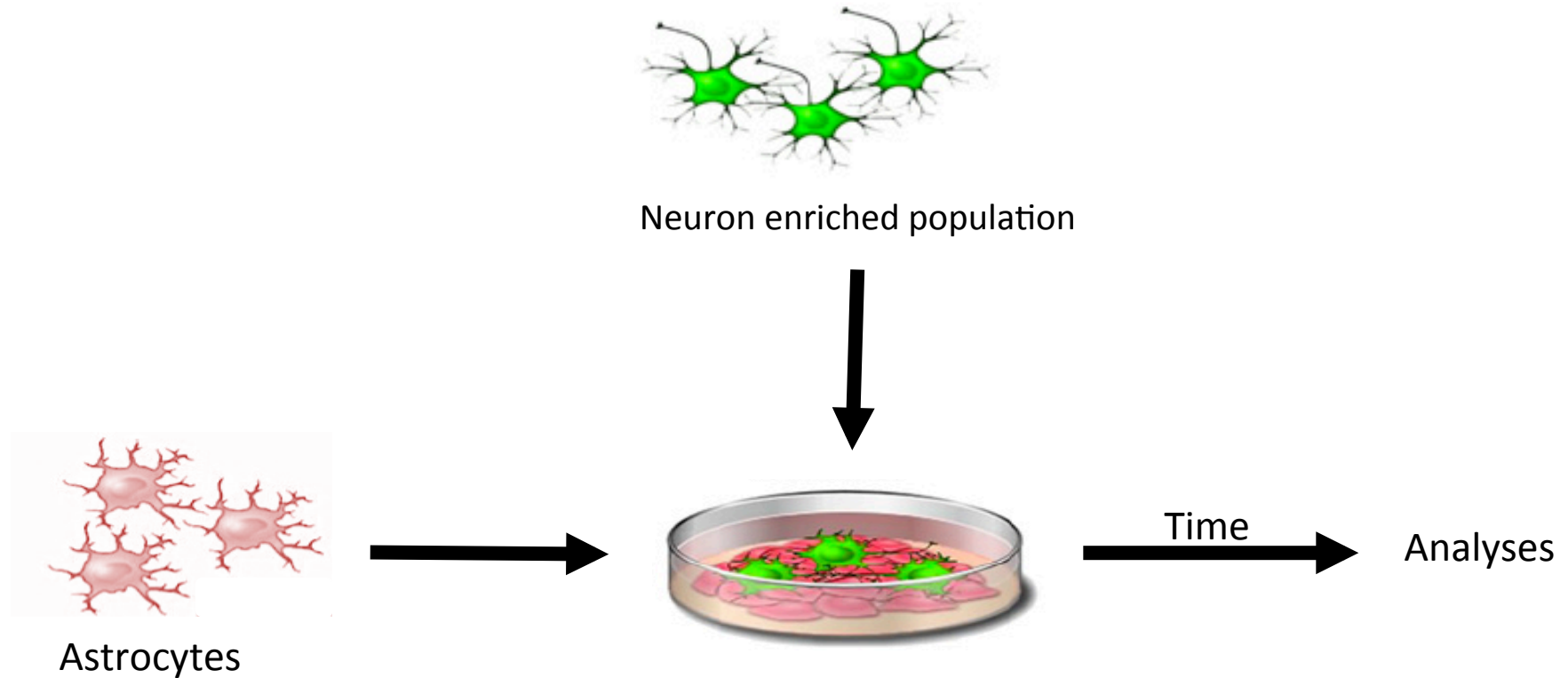
3s



ASD



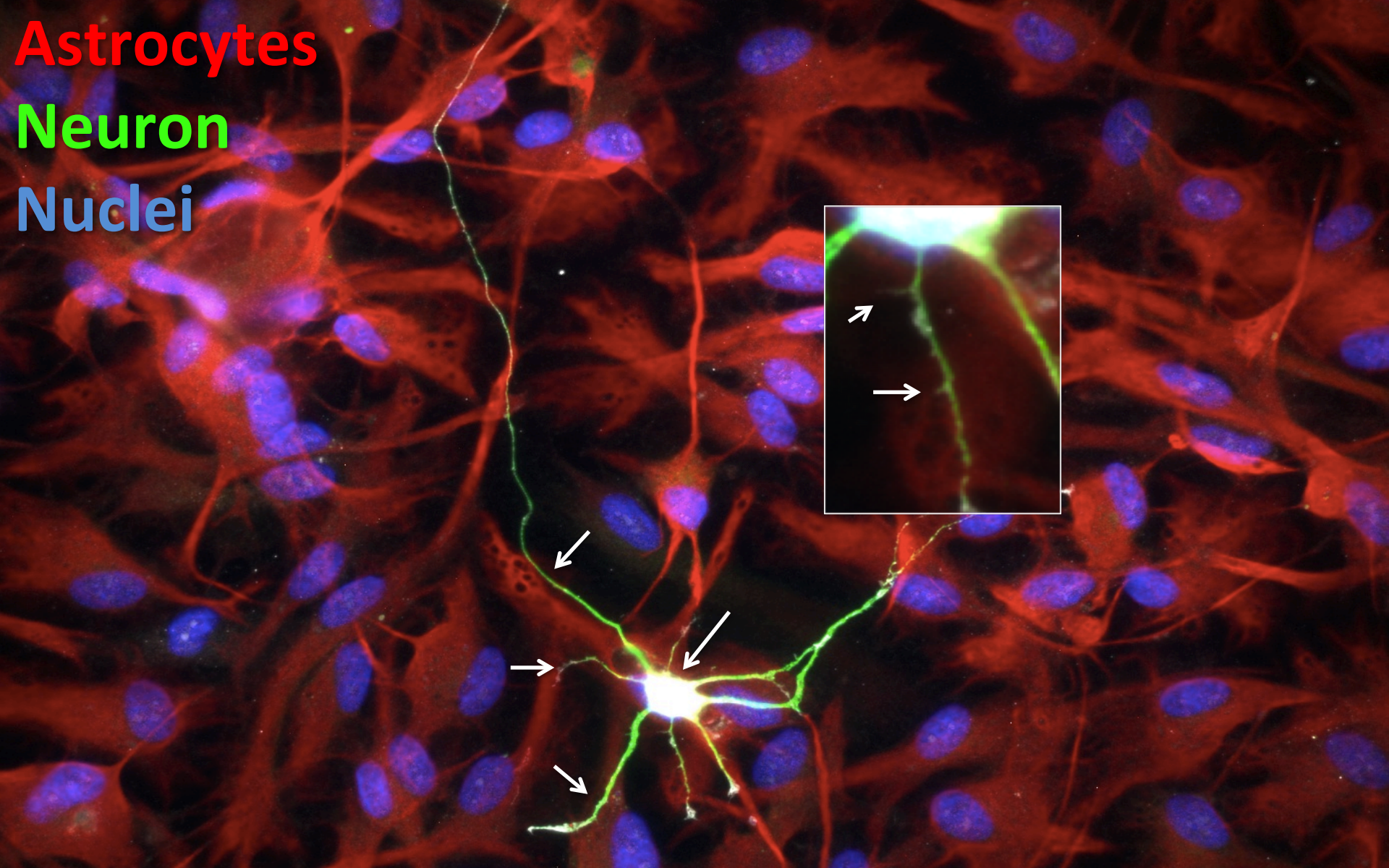
Effect of ASD astrocytes on human neurons



Astrocytes

Neuron

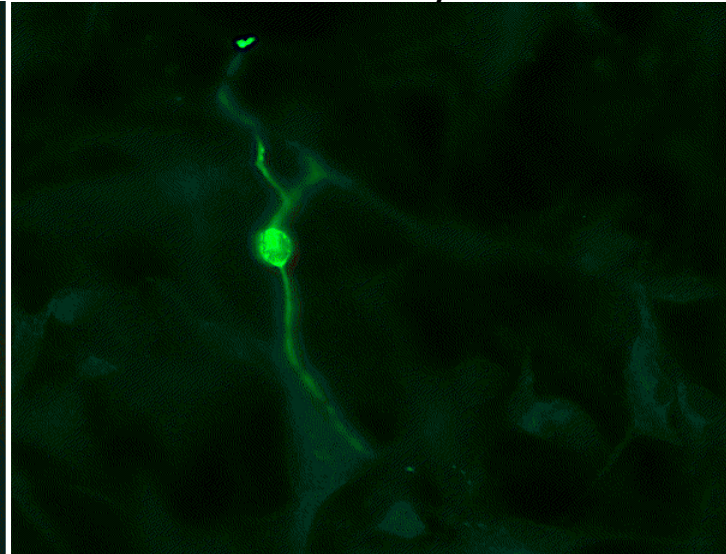
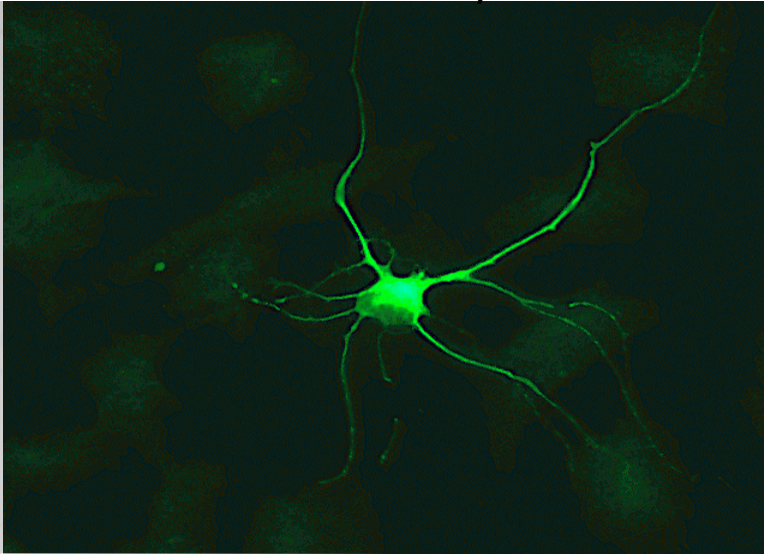
Nuclei



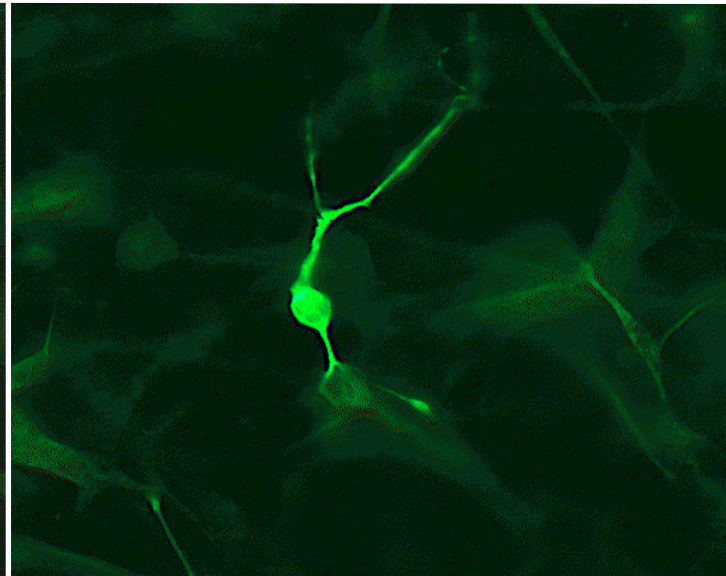
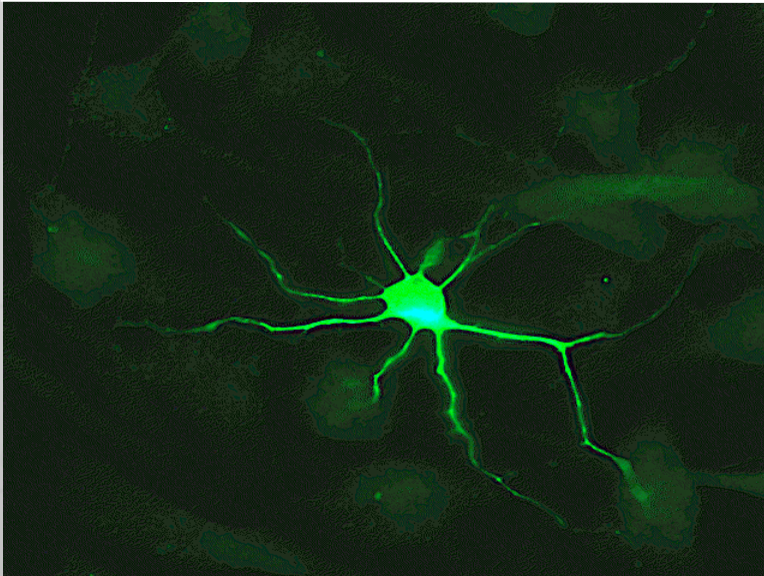
WT Astrocytes

ASD Astrocytes

WT Neurons



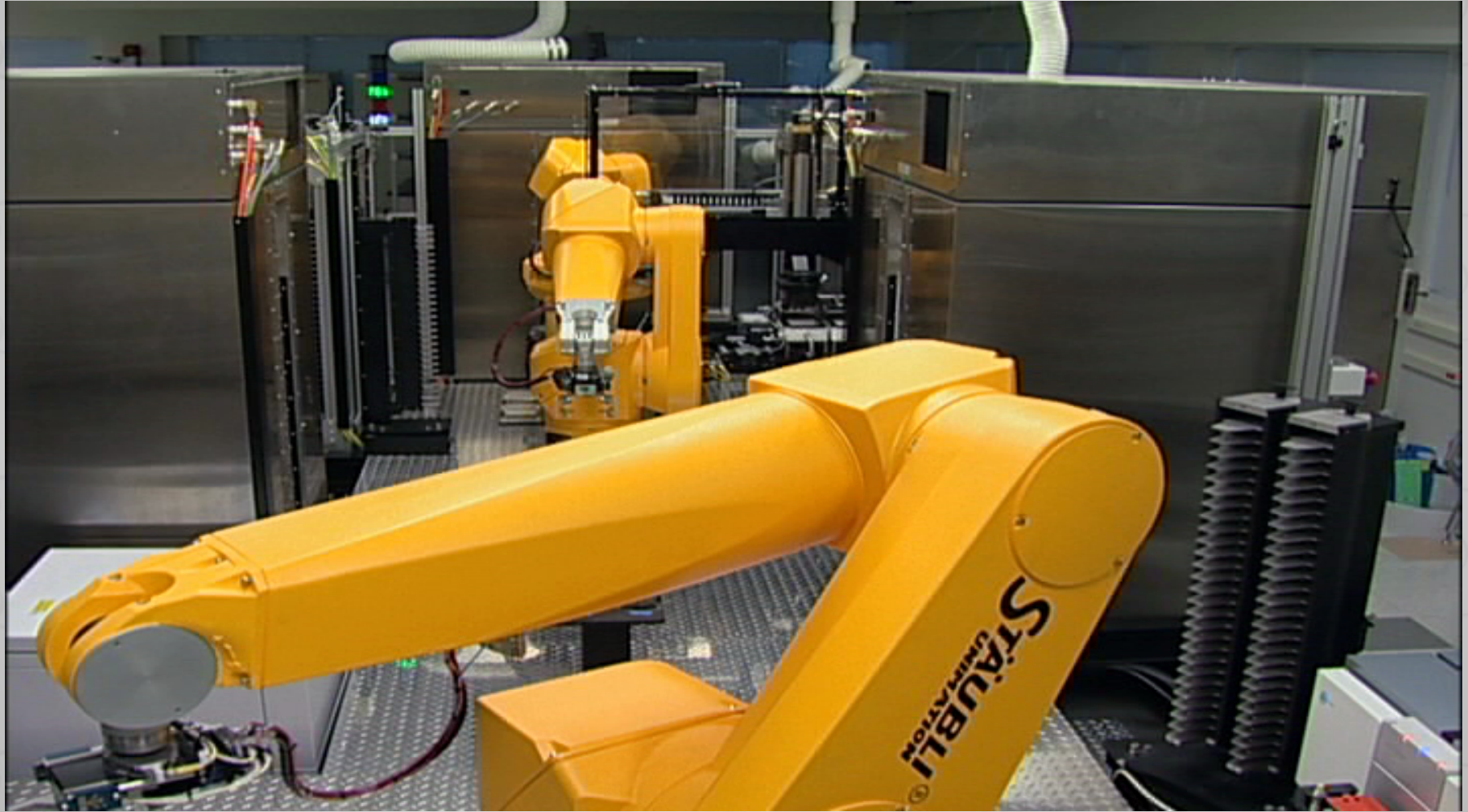
ASD Neurons



3 years, 3 drugs



Translational facility at NIH



“We always overestimate the changes that will occur in the next two years and underestimate the changes that will occur in the next ten. Don’t let yourself be lulled into inaction.”

Bill Gates



NIH
CIRM
IRSF
IFCR



**HUMANE SOCIETY
INTERNATIONAL**

Families and patients



THE MUOTRI LAB

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