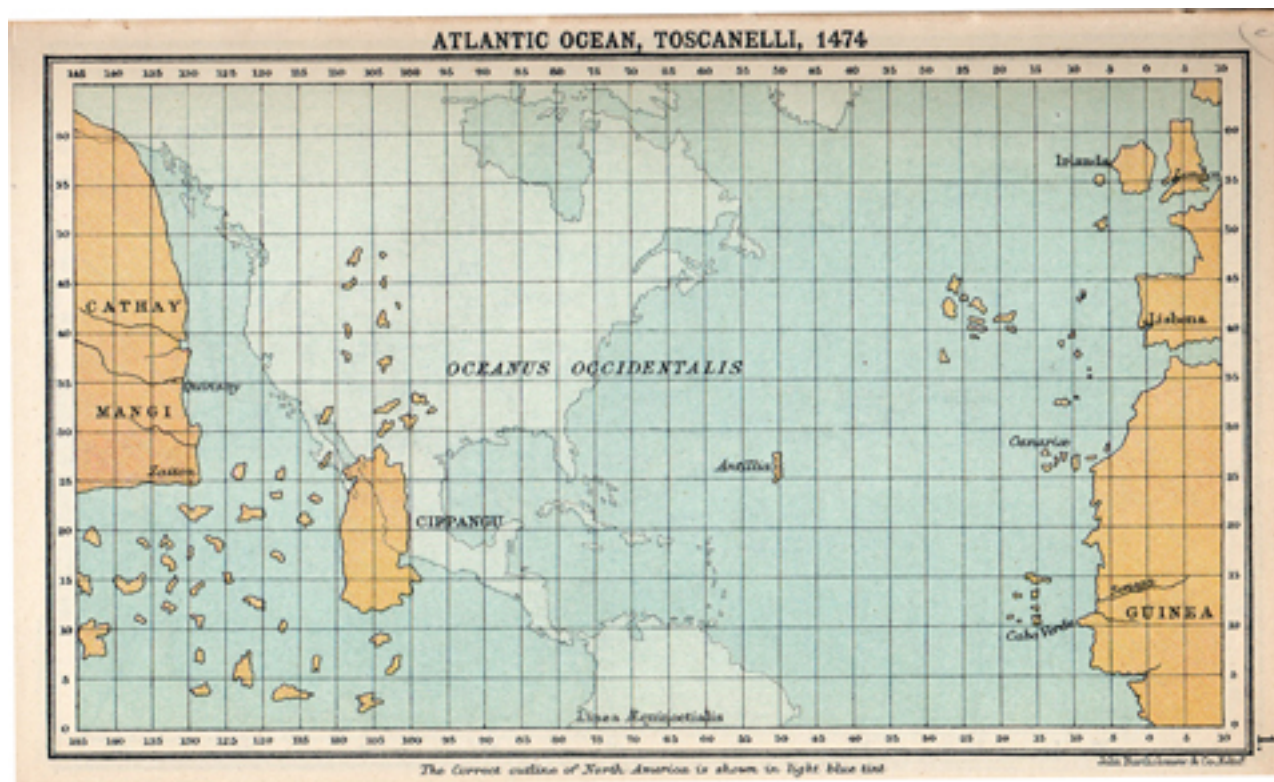


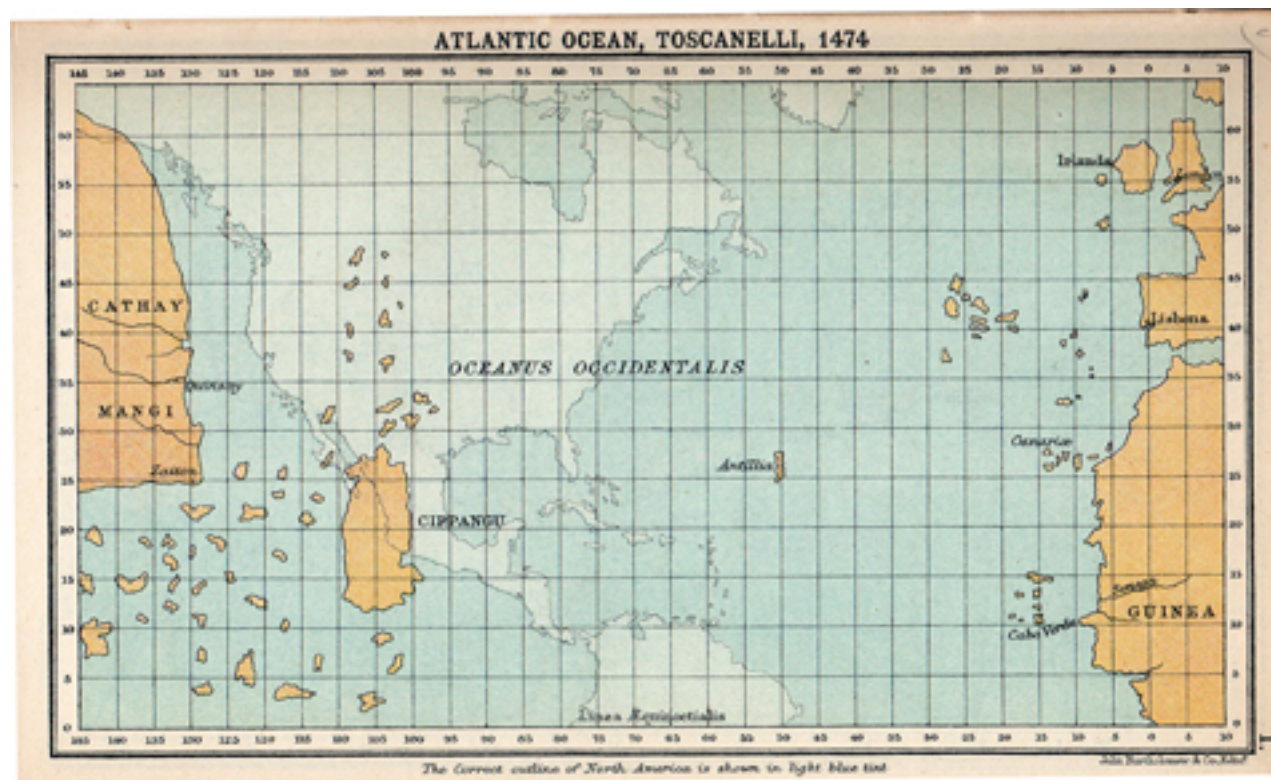
NEWS

Uncharted territory

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Last year, Pfizer became the first major pharmaceutical company to set up an **autism research unit**, as we reported. The idea didn't come from top executives looking at the bottom line. It grew out of a grassroots effort by a group of Pfizer employees with personal connections to autism.

At the **Autism Consortium Symposium 2010** in Boston earlier this week, **Robert Ring**, director of the new unit, says that the reason companies are sitting on the sidelines is because autism and other neurodevelopmental disorders pose unique challenges to drug developers.

First, there is no roadmap for developing a drug targeting the central nervous system in children because no one has done it before. In fact, there is little precedent in getting drugs approved for children, period.

Then too, most pharmaceutical companies don't have a pool of employees with experience in neurodevelopmental disorders. "You can't readily find people with this kind of expertise," he noted. Add to that the uncertainty about drug targets, lack of animal models and, on the business end, gaps in understanding the epidemiology of autism and the potential size of the market for autism

drugs.

Finally, though it is clear that "the synapse is the smoking gun," in terms of mechanism, it remains unclear how to translate that into therapeutic development.

Autism is much more than just its core set of deficits — social and language impairments and repetitive behavior — Ring pointed out. Sleep and mood disorders, anxiety and attention deficits are also associated with autism and widen the therapeutic possibilities, but also add to the uncertainty about which symptoms and pathways should be targeted.

Children with autism take a raft of psychotropic drugs. Their use is strictly off-label however; none of the various antidepressants, antipsychotics, stimulants and anticonvulsants prescribed to people with autism has been evaluated for efficacy or safety in this population. And some have unpleasant side effects, including drowsiness and weight gain.

Drug companies are always interested in recycling research from overlapping areas —for example, schizophrenia — and many may already have compounds on the shelf that treat the symptoms of autism.

Still, only one company has a drug in the works that targets core features of autism. **Cypress Bioscience** is refining an intranasal form of the drug is carbetocin, a form of oxytocin, to treat the social withdrawal and repetitive behaviors that are among the hallmarks of the disorder.

Collaborative partnerships between academia, biotech, nonprofits and Big Pharma are more important than usual in the field of autism therapeutics, says Ring. "We should be agnostic about approaches," he said, "but religious about the data."

For all reports from the Autism Consortium Symposium 2010, [click here](#).