

In promising news, the United States [Food and Drug Administration](#) is holding [two days of panel hearings](#) this week to address the challenge of how to get biotech and pharma companies more engaged in developing drug treatments for orphan conditions - defined as any condition affecting fewer than 200,000 Americans. With estimated US statistics of around 100,000 individuals affected by NF1, 12,000 by NF2 and 7,500 by schwannomatosis, the forms of neurofibromatosis fit well within this cap.

All told, one in ten Americans is affected by one of more than 6,000 orphan diseases, so this is a population that cannot be overlooked forever. There has been a great deal of talk from a number of companies such as [Pfizer](#) and [Novartis](#) in the last few months expressing an interest in rare diseases - but this has yet to translate into real action. Neurofibromatosis has the added challenge that the condition can affect individuals in many different ways - ranging from a variety of nerve tumors, to bone dysplasia, to learning disabilities - limiting patient numbers in any demographic even more. It is hard to engage companies in neurofibromatosis and other orphan conditions because the profit margins are likely to be very small at best. The Children's Tumor Foundation works to break down these barriers through programs like our NF Preclinical Consortium and Drug Discovery Initiative, which have attracted and engaged companies like Novartis, Genentech and Avila Therapeutics with positive preliminary results. That said, the real challenge will come when a potentially effective drug is identified and needs expanded to as large scale clinical trial - something for which we'll need the backing and heft of industry. Only the FDA can hold industry's feet to the fire on this matter. So we'll watch the outcome of these panels closely.

In other news, the [National Institutes of Health](#) has also been pushing for advancement in orphan conditions in recent times. Now they are [promoting 500 licensing opportunities](#) in NIHs possession, which have potential applications for orphan conditions, and which they'd like to license out to interested parties. The Foundation will endeavor to learn if any of these offerings are related to neurofibromatosis and see if we can facilitate their advancement.