

Traditional clinical research paradigms relying solely on brick and mortar in-person engagement between researchers and patients with rare disease are geographically sparsely distributed globally, are already burdened by the disease they carry, often genetic and debilitating, are often dependent on a caregiver to take paid time off to support them, yet are still willing to travel across the globe to access a life-saving or altering treatment options such as gene therapies. But it is unfair to expect them to do so when it is really not necessary in this day and age of Telemedicine, Digital Health, and wearable devices. Unique to rare diseases R&D are patient registries and natural history studies. These multi-year studies are oft