

# ANATOMY OF A NEXT GENERATION RARE DISEASE PATIENT ADVOCATE

## EARS:

### Listening to those who have come before

There are many learnings to take from those who have come before. There's no need to reinvent what already works. Finding a mentor who will guide you is the fastest path to progress. It's essential to learn not just what to do from your mentors but benefit from their mistakes to avoid making them yourself.

## NOSE:

### Sniff out others with the condition

There is strength in numbers. It will be essential to find other patients with the same condition to start understanding a rare disease, attract researchers, and ultimately engage drug developers in pursuing the condition.

## STOMACH:

### De-risk research in your disease

Pharmaceutical companies don't have a strong stomach for risk as rare disease advocates do when developing new therapies. Find ways to de-risk research and eliminate barriers to attracting a biopharmaceutical company to pursue a therapy.

## GUTS:

### Currency is currency

Data may be the new currency, but money still works well. Successful advocates overcome any shyness they may have about making an ask. All it takes is some guts.

## DNA:

### Leverage commonalities with related conditions

Despite different underlying genetic causes, many rare diseases share common phenotypes. Given the small population for many rare conditions, there's increasing recognition of how similar diseases with different genotypes can help elucidate each other and advance research into their own condition.

## FOOT:

### Change regulators thinking

Patient advocates can change the thinking of regulators and motivate drug developers and researchers, so they share their feeling of urgency. Sometimes a good swift kick in the right place is all it takes.

## BRAIN:

### Become a citizen scientist

As you gain an understanding and learn from your own experience and the experience of others, you may begin to see things about your disease that researchers do not recognize. Don't be afraid to gather and analyze data to help expand the understanding of your disease and help identify key symptoms that could point to potential treatments.

## EYES:

### It takes a scientist with vision

Find a scientist willing to embrace challenges, share a patient advocate's sense of urgency, and reimagine what is possible.

## CHIN:

### Become the expert on your disease

Despite the lack of formal scientific training, next-generation advocates frequently engage with scientists, read journal articles, and compare notes and observations with other patients and parents to make themselves experts in their disease. Learn to stroke your chin in a contemplative way to help people understand you are an expert in your disease.

## HEART:

### Have compassion for those who follow

Rare disease advocates often describe themselves as having a membership in a club they would never have wanted to join. However, for next-generation advocates, regardless of whether their efforts have saved the life of a loved one or they have suffered a heartbreaking loss, their advocacy efforts don't end. They mentor others, drive organizations forward, and work to ensure others who come behind them have treatments. For these advocates, it is a membership for life.

## ARMS:

### Leverage technology

Rare disease advocates often have to do the heavy lifting without the benefit of a lot of resources. However, technology is changing the face of rare disease from diagnosis to treatment. Find ways to leverage technology to do more with less.