



# Good, Better, Best: Envisioning the Future

Connie Lee, President and CEO, Angioma Alliance

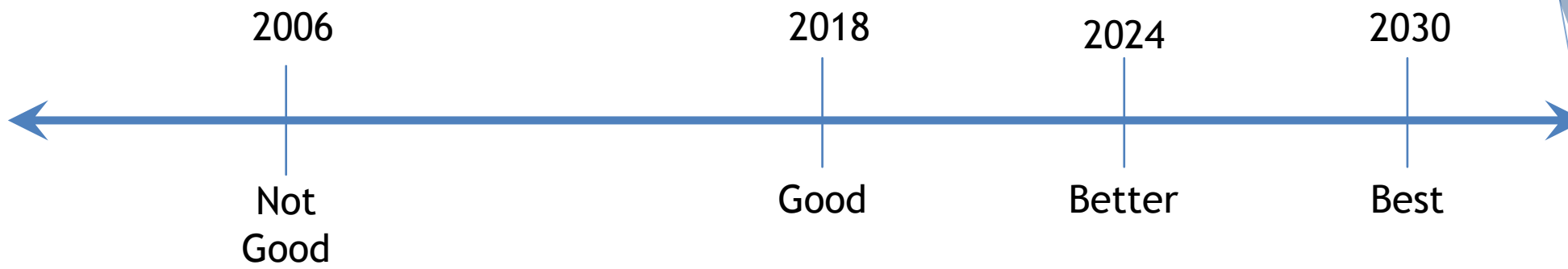
## Our Mission

To inform, support, and empower those affected by cavernous angioma and drive research for better treatments and a cure



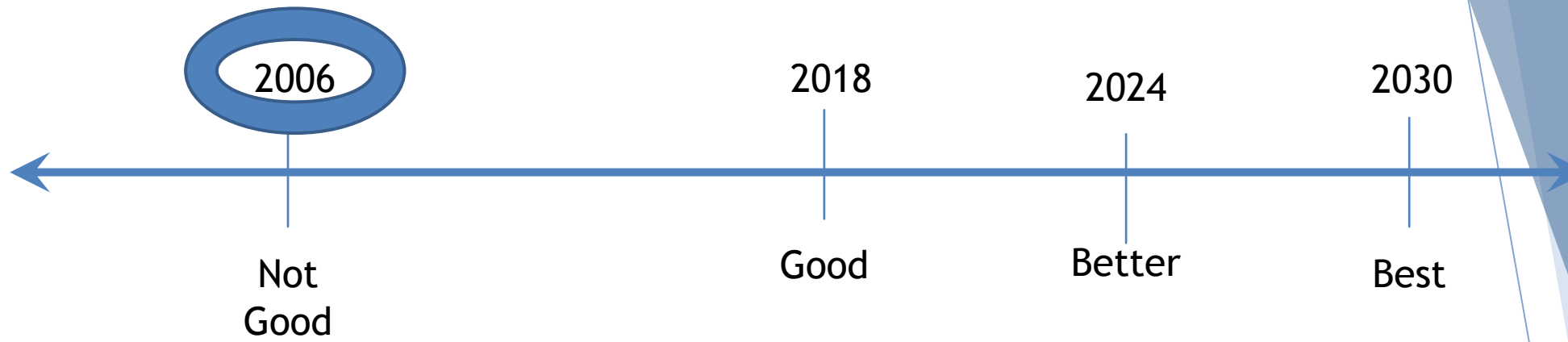
**ANGIOMA ALLIANCE**

because brains shouldn't bleed



## The Full Picture

- Diagnosis
- Tracking/Predicting
- Lesion Treatment Options
- Multi-Systemic Impacts
- Recovery/Restoration
- Comprehensive Clinical Care
- Supportive Community

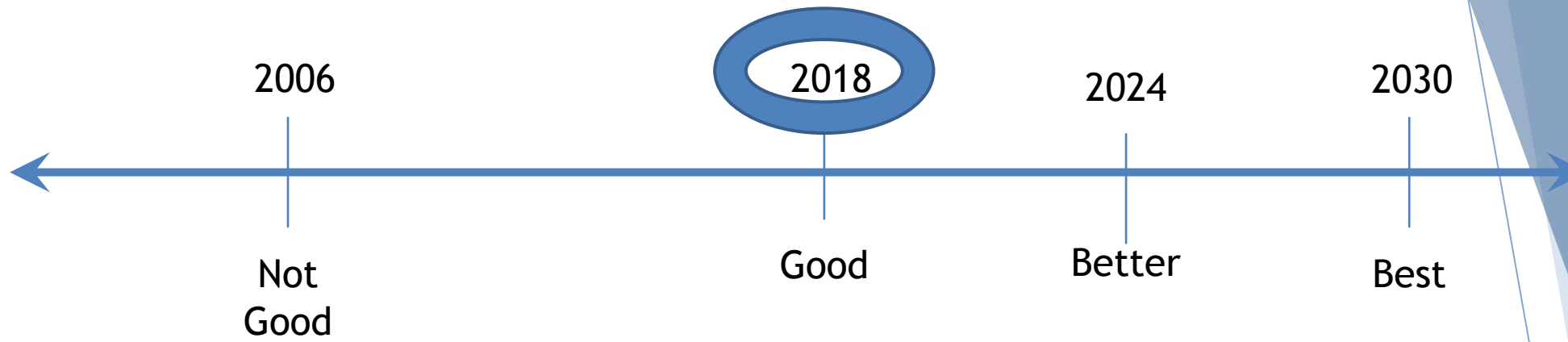


## 2006: Where We Were

- ▶ Inconsistent diagnosis
- ▶ Few natural history studies
- ▶ Surgery/radiosurgery were the only interventions
- ▶ What multi-systemic effects?
- ▶ Basic rehab
- ▶ What prevention?
- ▶ A few knowledgeable surgeons and fewer neurologists
- ▶ Loose patient community

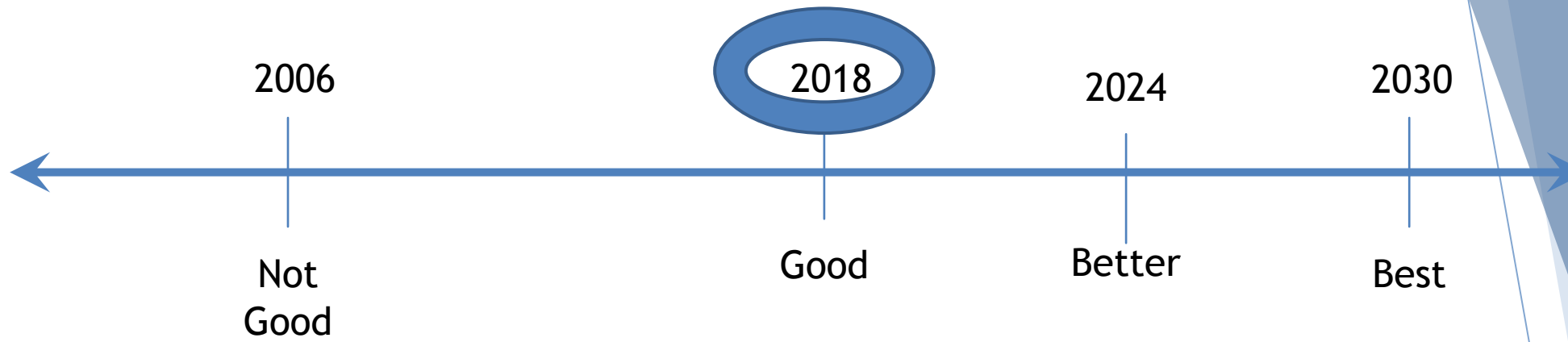


More questions than answers



## 2018: Where We Are

- ▶ Diagnosis - SWI MRI; genetic testing (Angioma Alliance)
- ▶ Course - Natural history data regarding hemorrhage, incidental lesions, genetics, proliferation.
- ▶ Treatment - Surgery, Minimally invasive surgery, Vitamin D
- ▶ Identifying multi-systemic issues and causes/consequences

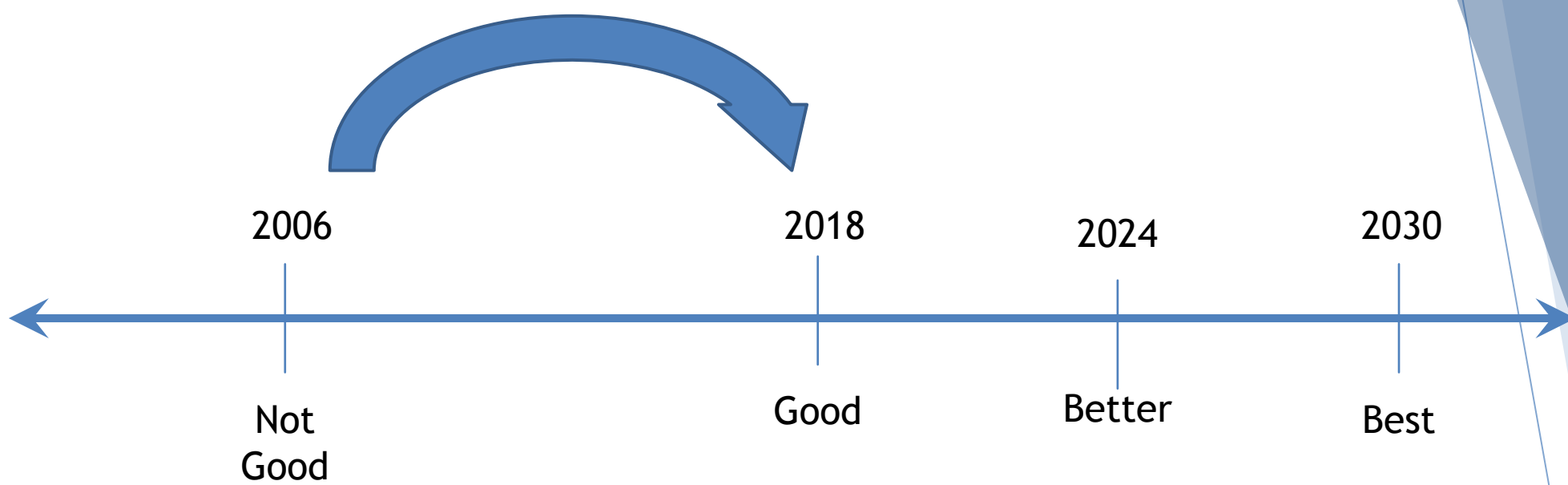


## 2018: Where We Are

- ▶ Rehab - More informed PT, OT, surgery, orthotics, robotics
- ▶ Prevention - Option of IVF with PGD
- ▶ Care - 7+ US Clinical Centers recognized for providing expert multi-disciplinary care
- ▶ Community - We can find each other, offer support, participate in advocacy/research/raising funds.







### How Did We Get Here?

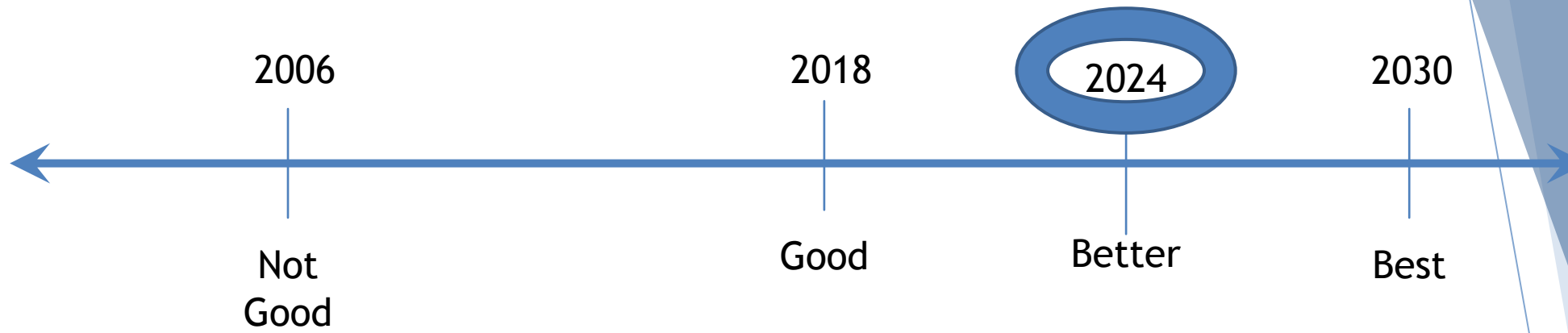
- ▶ Genes identified/testing
- ▶ Scientific Meeting to advance collaborations
- ▶ DNA/Tissue Bank
- ▶ Legislation and advocacy
- ▶ SAIVMS, BVMC Project and other natural history studies
- ▶ Centers of Excellence
- ▶ Community building



**You're  
Awesome!**

U.S. ARMY



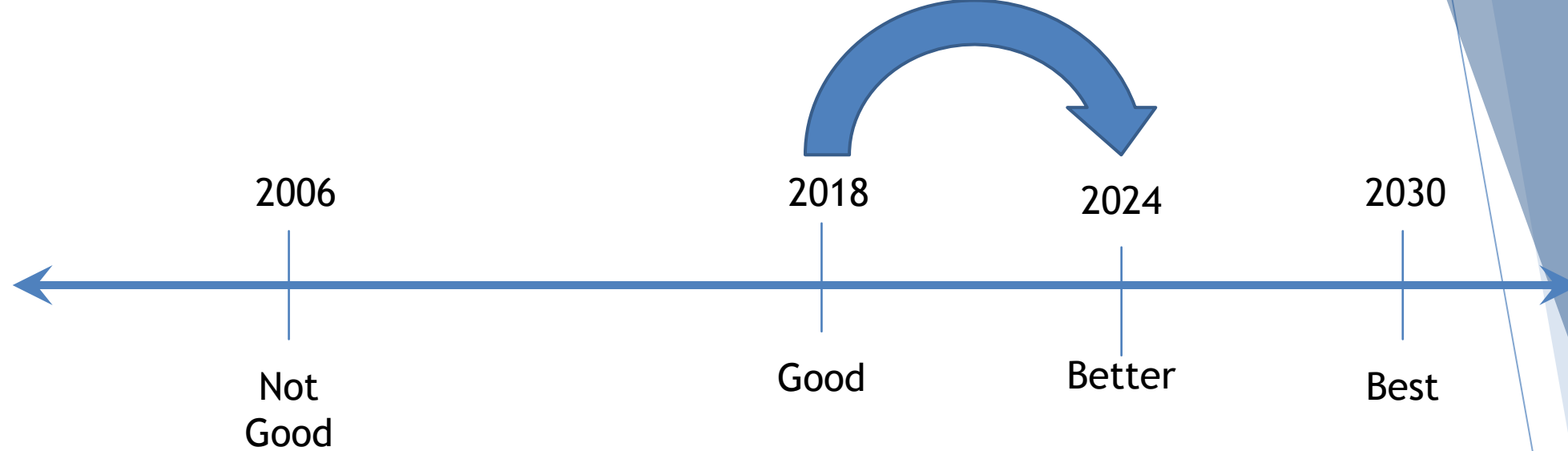


## 2024: Real Treatment Options!

- ▶ Restoration of lost function - stem cell therapy trials? (Stanford)
- ▶ Prevention using microbiome change via individual diet change (CCM diet)/transplants, cheaper IVF with PGD
- ▶ Many Clinical Centers providing expert care around the world
- ▶ A community that is organized and mobilized for research participation, fund and awareness raising, and outreach to clinicians/patients.

# Path to the Future





## How Do We Get There?

- ▶ Participate and/or support clinical trials and other clinical research: join patient registry, get testing, educate peers.
- ▶ Support basic/translational research: scientific meeting, advocacy, grants?
- ▶ Form many Community Alliances for outreach to medical community, etc.
- ▶ Increase international patient participation in research.

# Working Together Will Speed Progress!

Oceania: 38,000,000

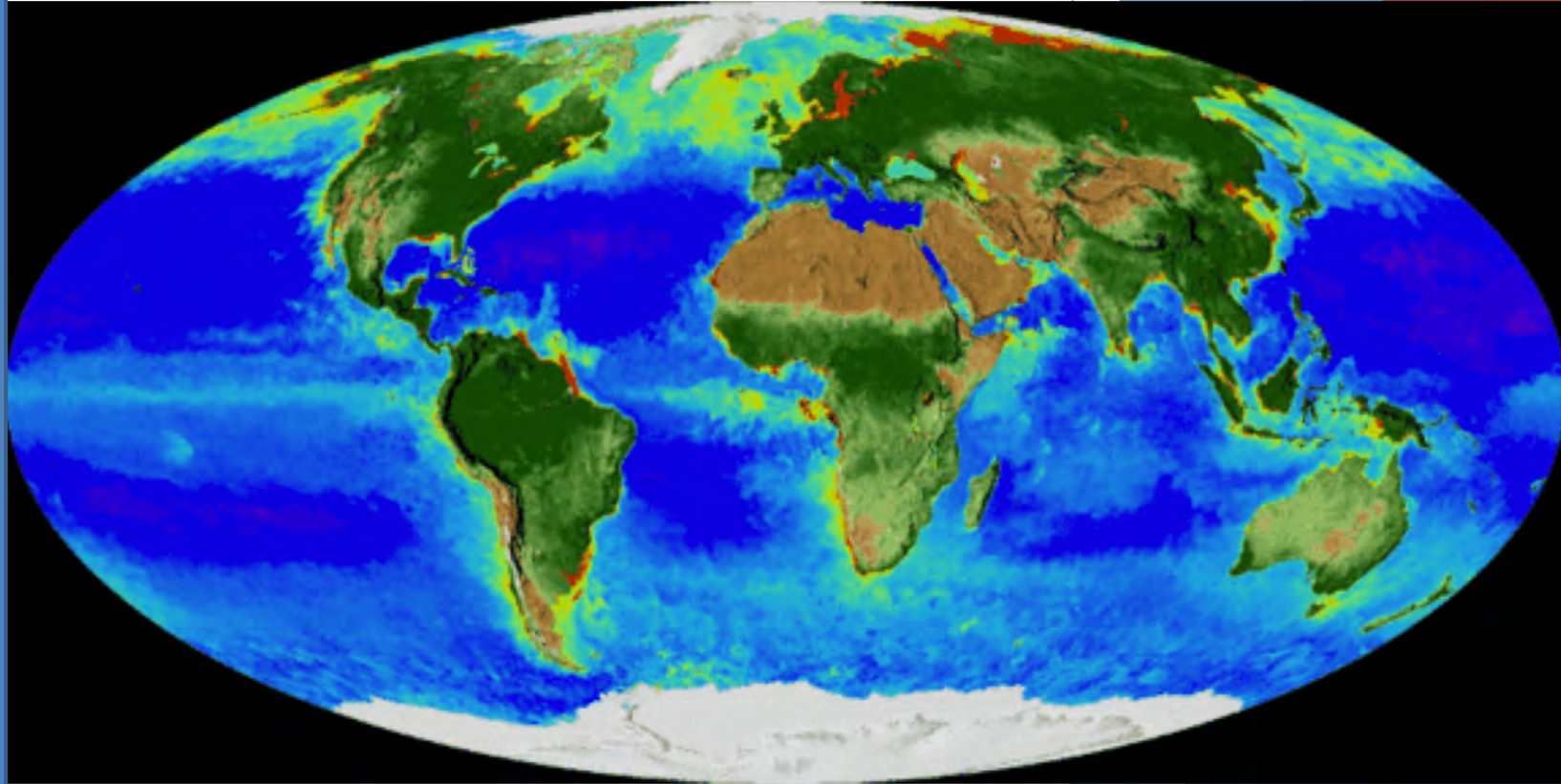
South America: 422,000,000

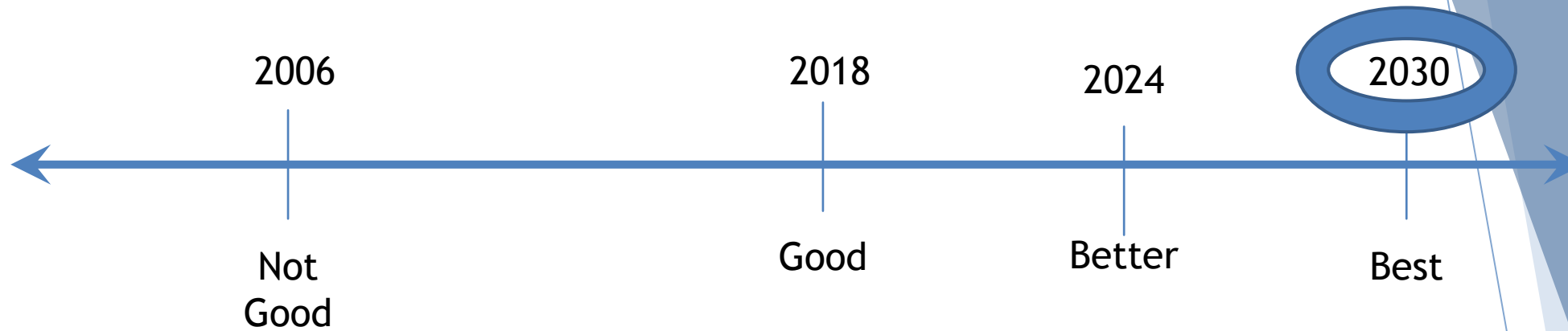
North America: 579,000,000

Europe: 741,000,000

Africa: 1,216,000,000

Asia: 4,463,000,000



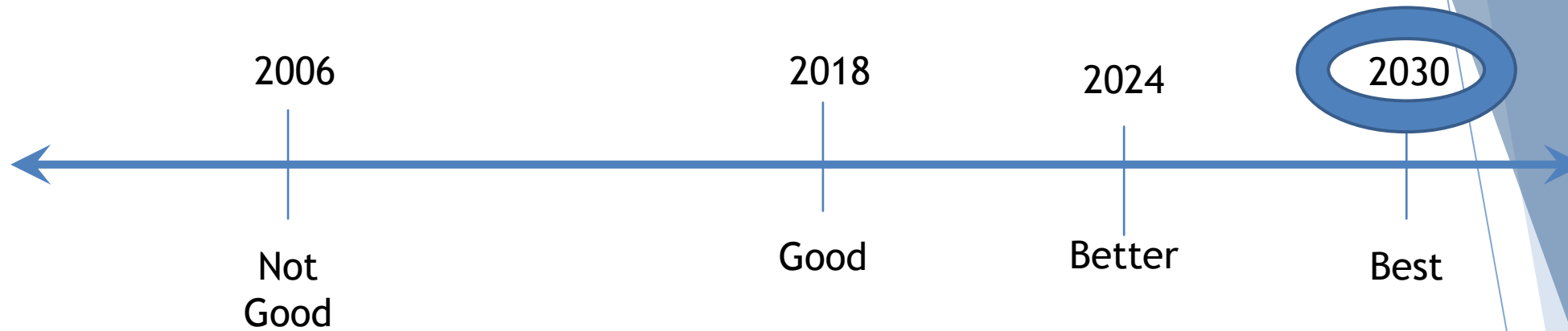


## 2030: The Final Destination

- ▶ Fast, cheap, and accurate diagnosis - urine biomarkers, \$50 genetic testing, \$200 10-minute MRI
- ▶ Fast, cheap, 100% accurate course prediction - blood/imaging biomarkers, something else?
- ▶ Obliterate lesions with no recurrence or side effects using a low-cost one-time medication or non-invasive treatment.

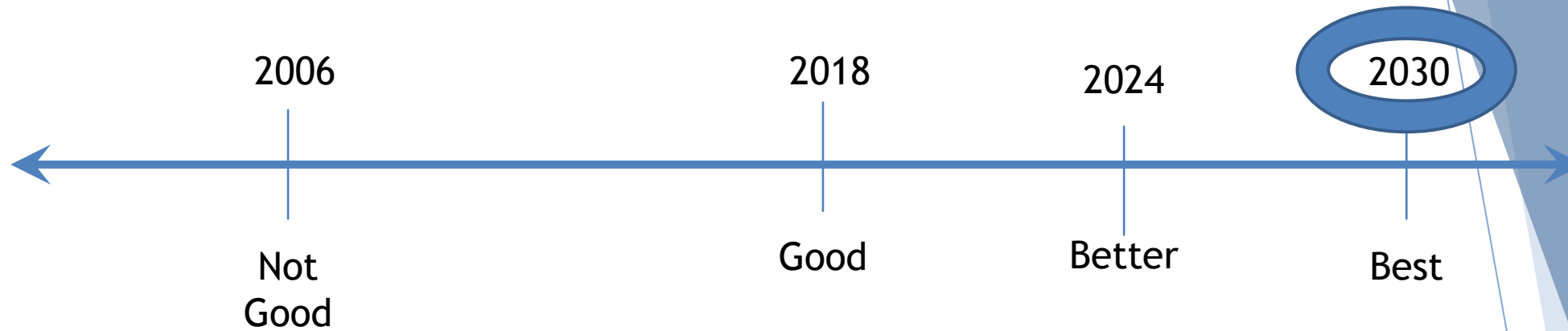






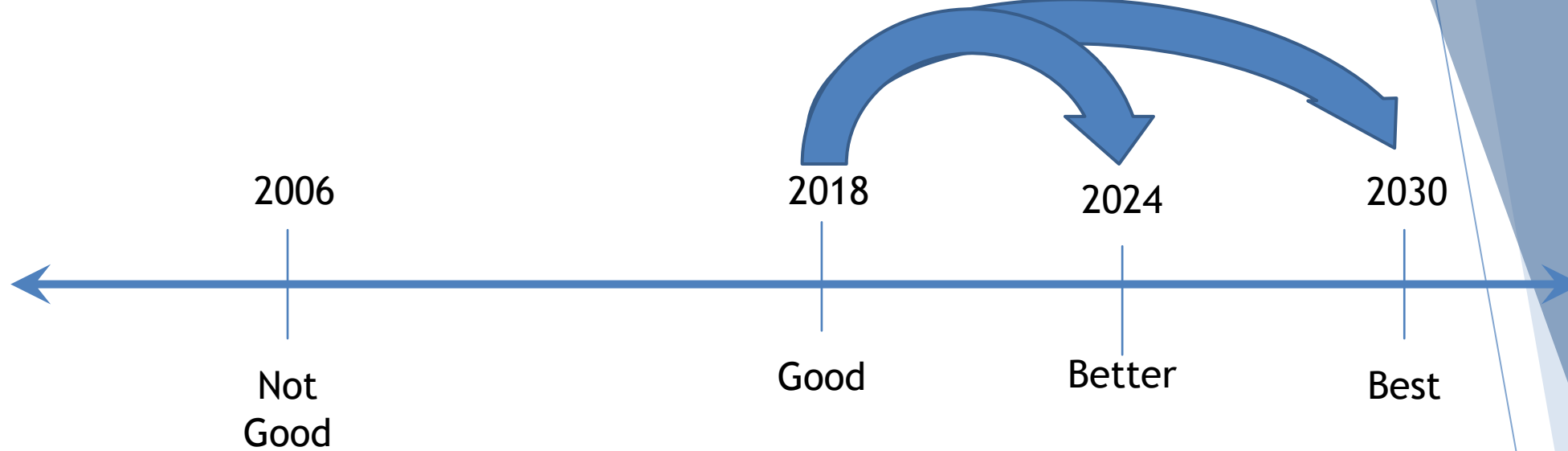
## 2030: The Final Destination

- ▶ Eliminate multi-systemic impact of hereditary disease
- ▶ Cheap restoration of lost function as well as development of functions that didn't have a chance in those affected as young children.
- ▶ Societal microbiome changes.
- ▶ Gene therapy for prevention of generational transmission.



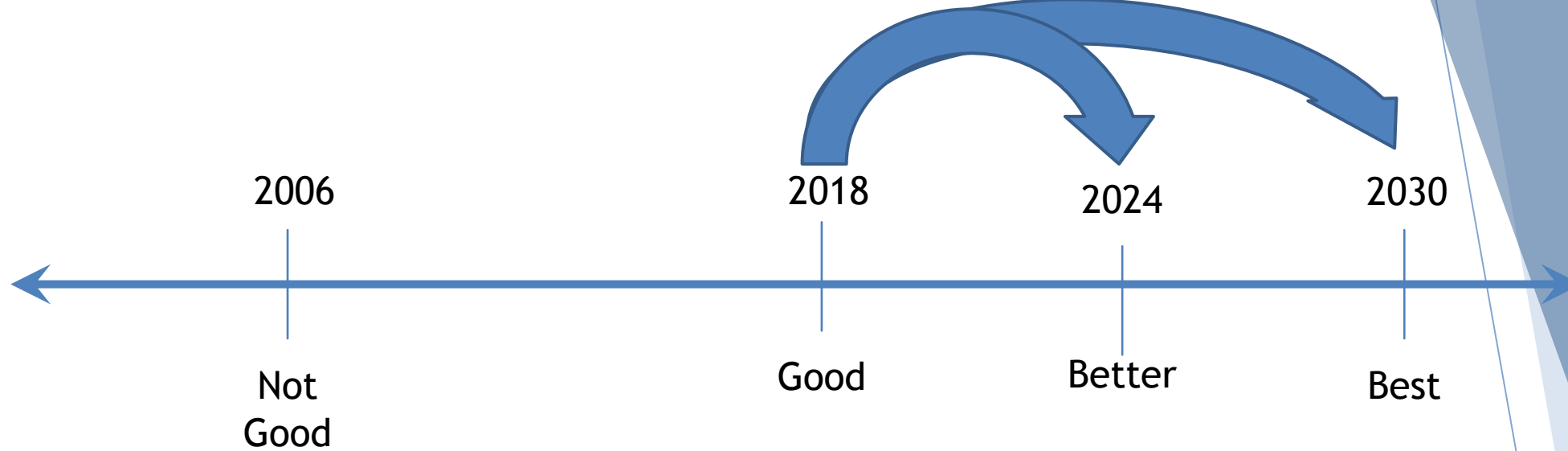
## 2030: The Final Destination

- ▶ Informed medical providers everywhere, even primary care.
- ▶ We will be known in all medical settings and among the general public.
- ▶ The cure, prevention, and restorative treatments will be readily available.



## How Do We Get To a Cure?

- ▶ Do all the same things we do for better treatments!  
Organize, mobilize, participate!
- ▶ Create a tandem, alternate route that supplements the pharmaceutical industry - just in case.



## How Do We Get To a Cure?

- ▶ Partner with other disease groups internationally whose illnesses may be amenable to same cure.
- ▶ Partner with governments, industries outside of pharma, and foundations.
- ▶ Fundraise to fill gaps in the development of cures.

# Take-Aways

- ▶ We are in a new era for cavernous angioma.
- ▶ This era will include better prediction, non-surgical treatments, informed care, and mobilized, supported patients.
- ▶ We are moving forward because of YOU and those around you!