# Search of Better Drugs for AHC: An Overview

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# Definition of Precision Therapy

- Approach for disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle for each person.
- In AHC this is centering on understanding the implications of the genetic mutations on the physiology with the subsequent aim of developing targeted therapies

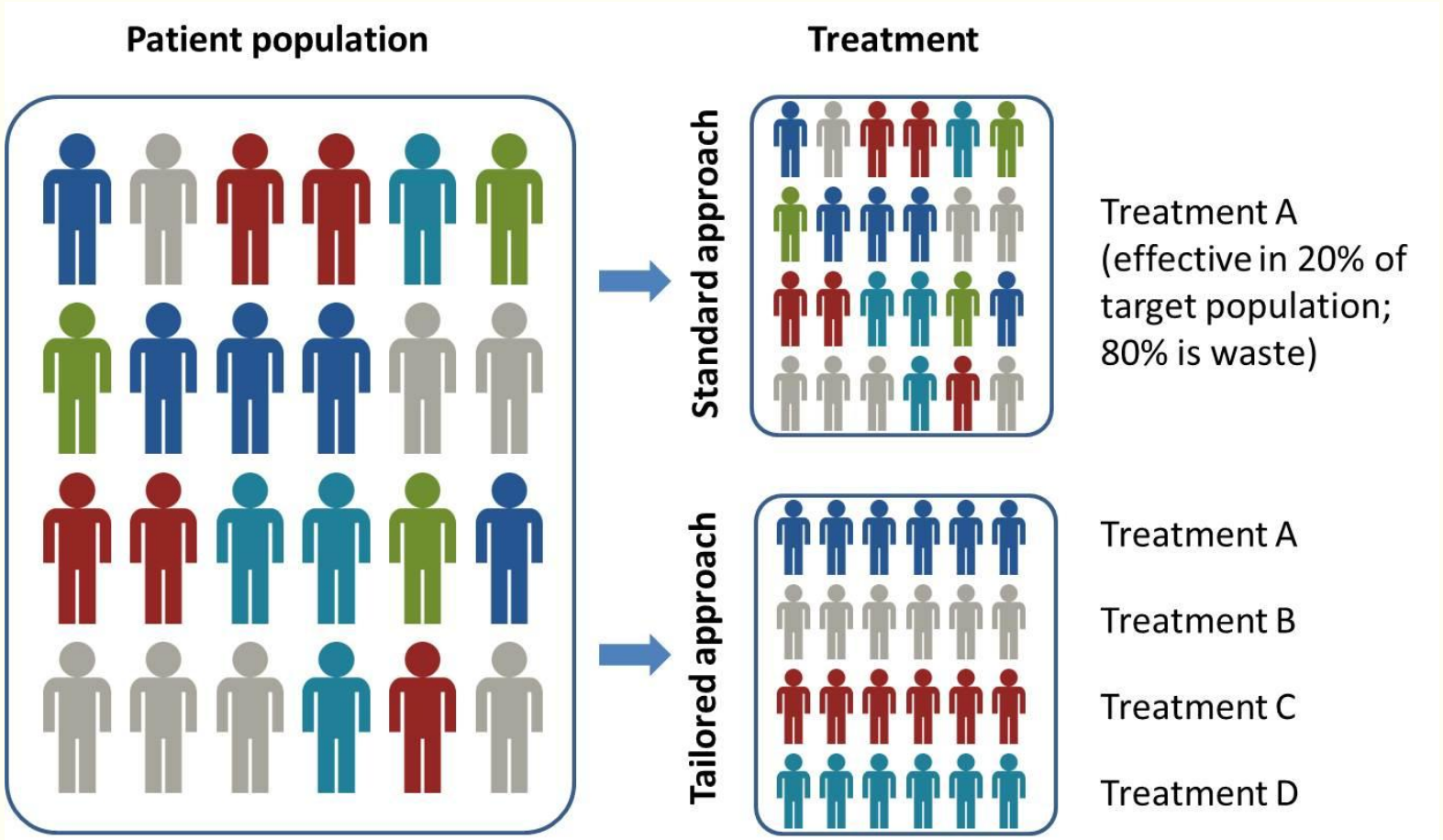
# Basic Science Aspects of Developing Precision Therapy

- Cellular models from heterologous expression in Xenopus laevis oocytes or mammalian cell lines
- Neuronal cultures extracted from the mouse brain or differentiated from iPSC lines derived from patients' somatic cells
- Organoids grown from stem cell-derived neurons which may be used to study cell proliferation and migration, in vitro.
- Genetic animal models behavioral and EEG alterations
- Each model can be studied independently or start with simpler and develop toward more complex models

# Clinical Aspects of Developing Precision Therapy

- Genetic assessment comprehensive
- Classification according to physiology
- Biomarkers for diagnosis and follow up
- Treatments specified to a person's molecular drivers.

# Potential Future Outlook for Novel AHC Therapies



# Ongoing Studies in Search Novel AHC Therapies

- Structural biology of ATPase
- Heterologous expression in oocytes and cell lines
- iPSC studies
- Neuronal cultures and Microelectrode Arrays
- Mouse Model studies
- RNA expression studies and in-silico analysis (Duke, MGH)
- Human ad-hoc and prospective controlled trial (triheptanoin)
- Most important in this process: the partnerships between family associations with the basic scientists and clinicians

# Conclusion:

The Future Holds a Lot of Promise for AHC Patients

Thank you for you Attention