

SBMA Patient Day 2022

10/07/2022

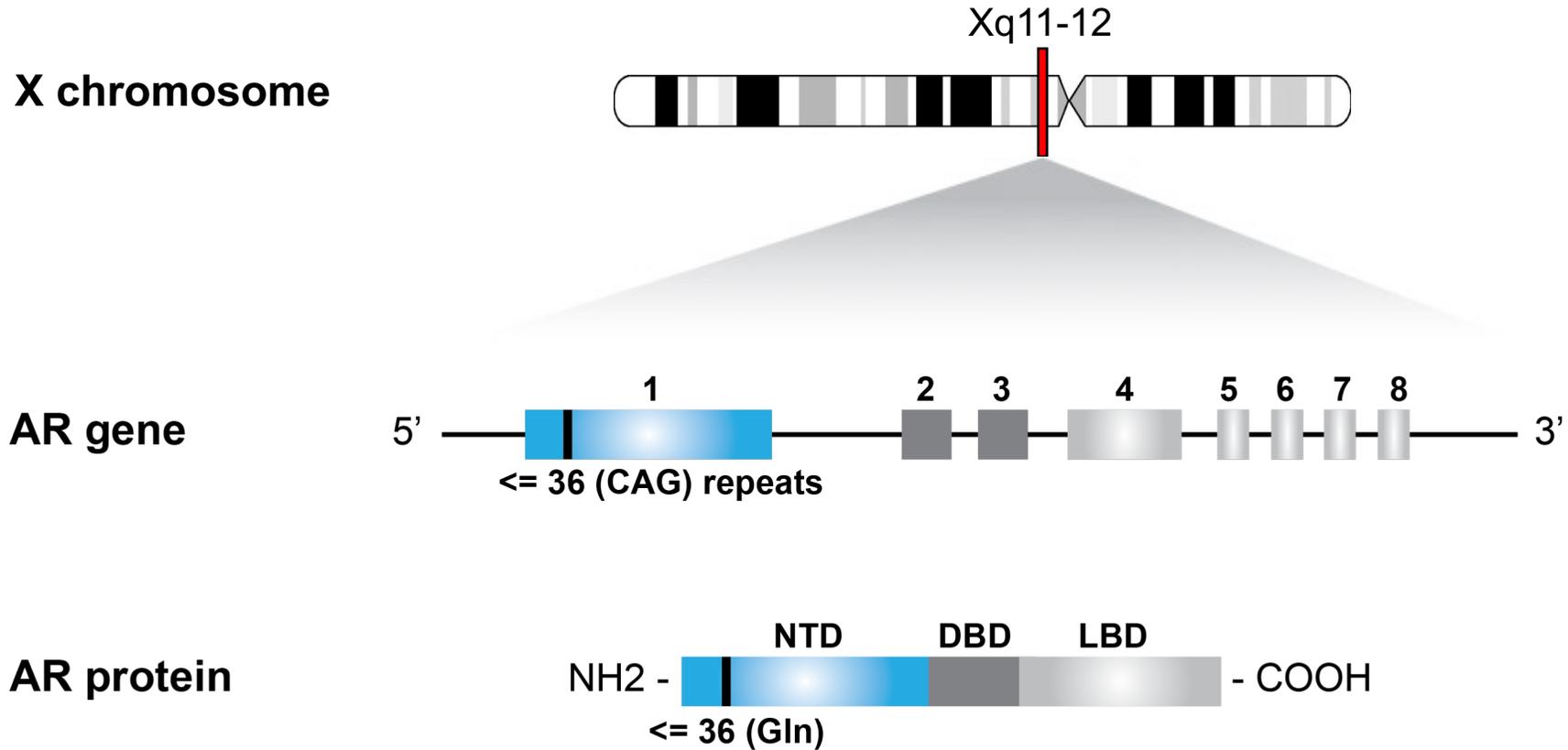


Carlo Rinaldi, MD PhD
University of Oxford

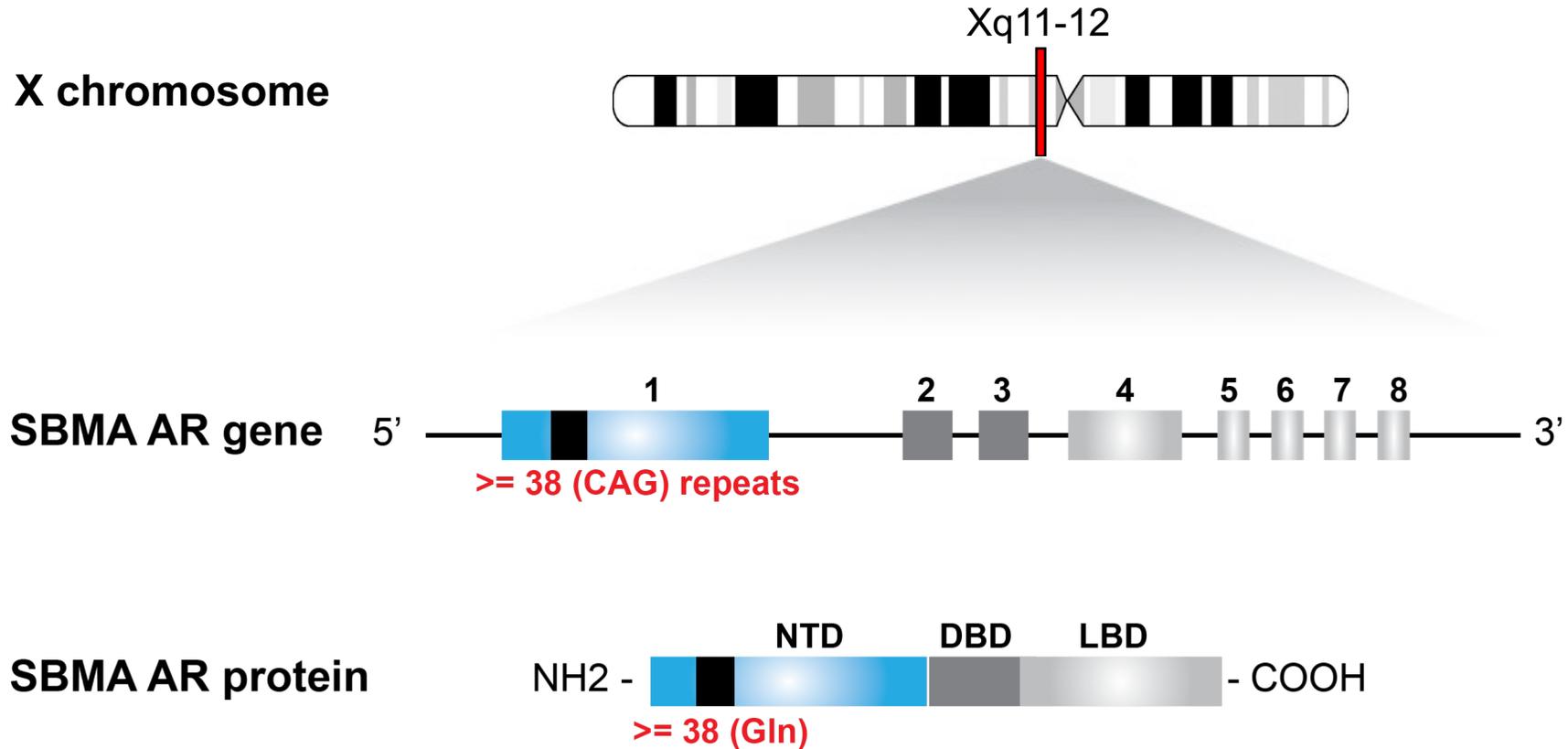
 @rinaldi_ca



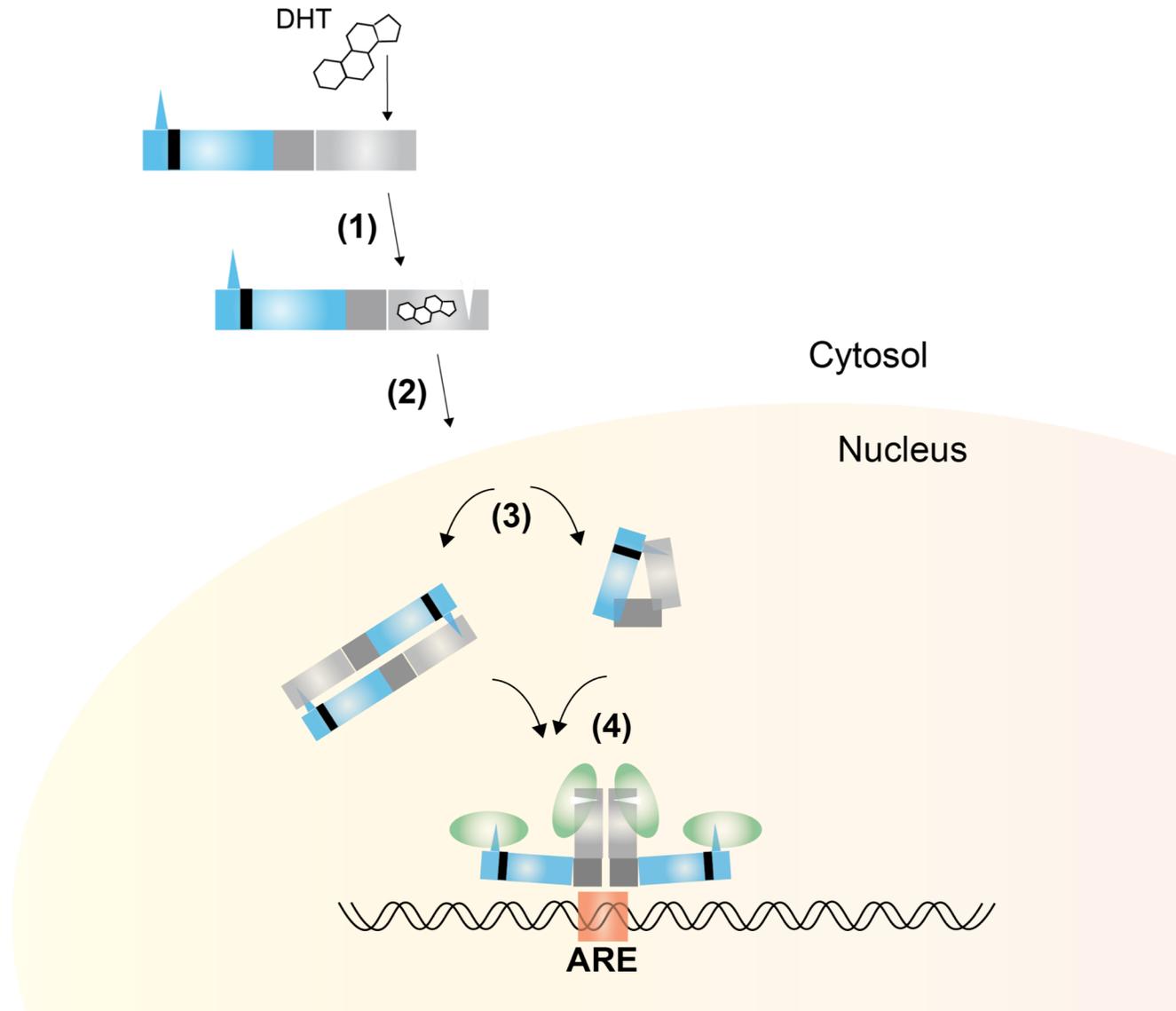
CAG repeat expansion – pathogenesis of SBMA



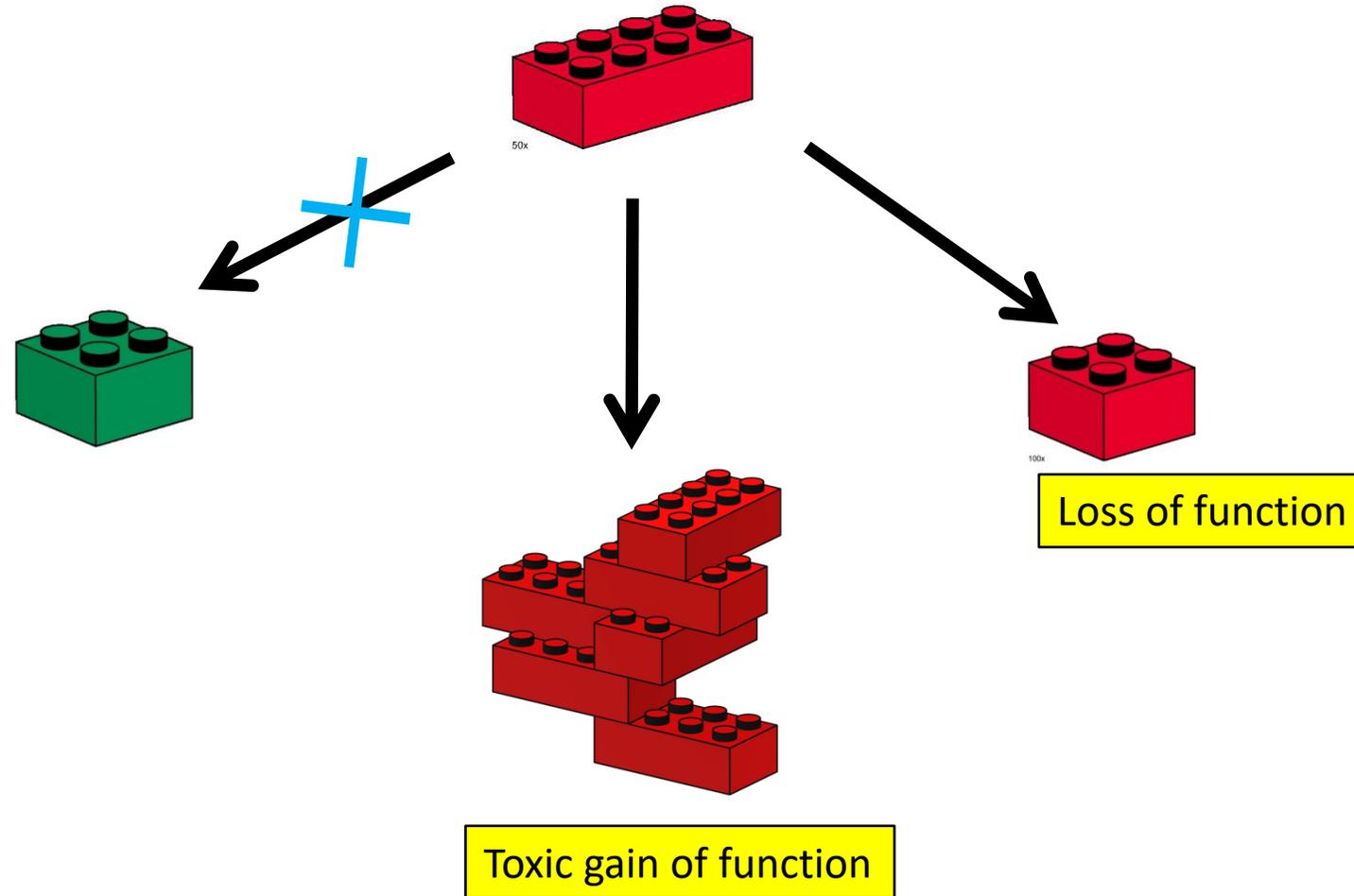
CAG repeat expansion – pathogenesis of SBMA



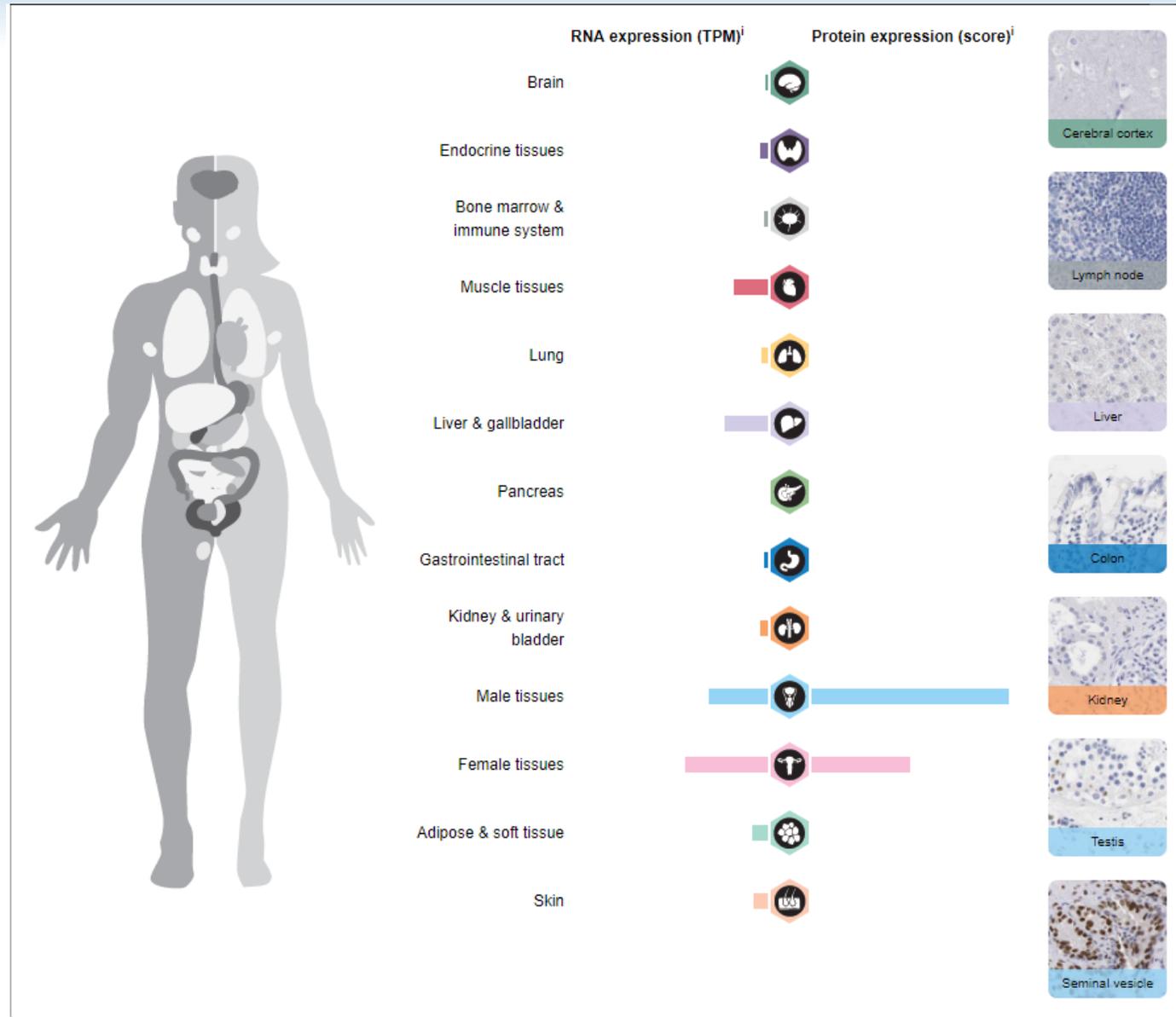
Androgen receptor as a ligand activated transcriptional regulator



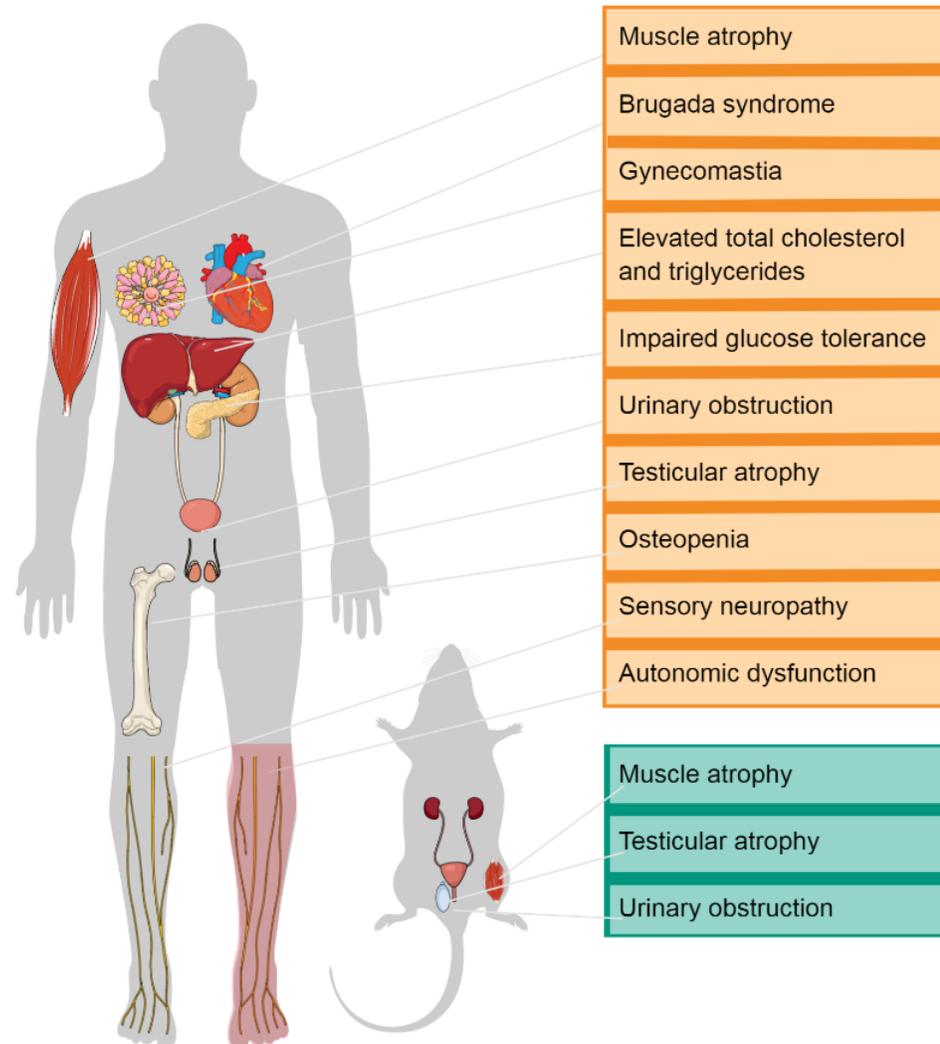
Mechanisms of pathogenesis



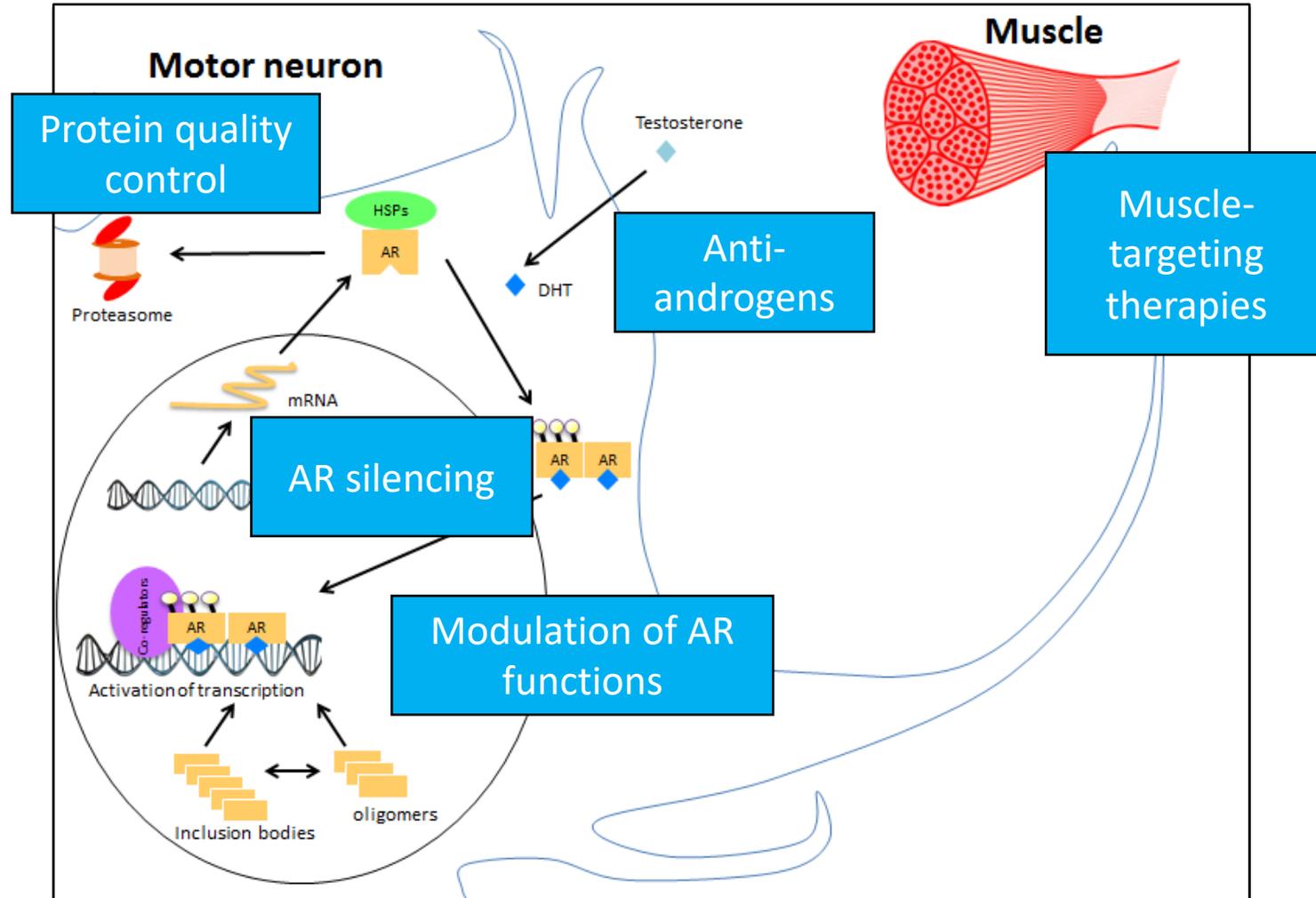
AR RNA and protein expression



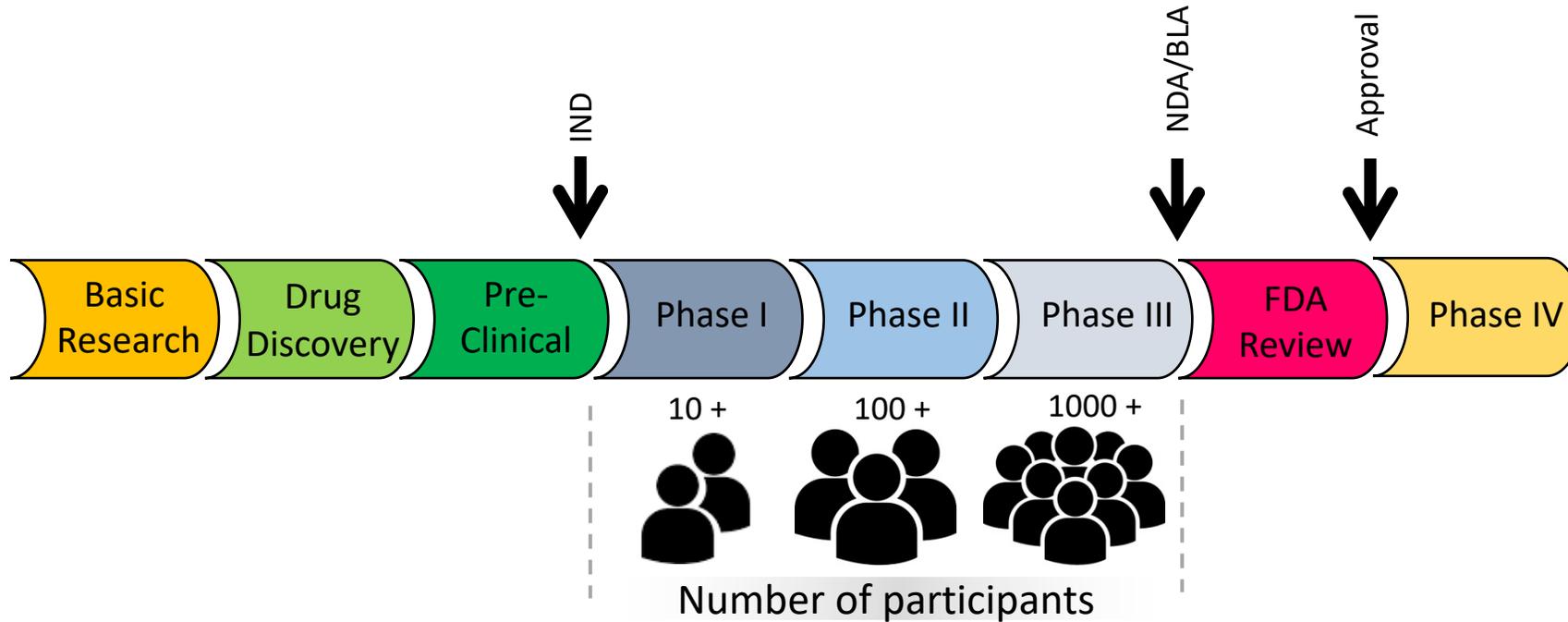
Extra-neurological features in SBMA



Treatment opportunities



Drug development process



Products



Average cost: \$1 billion +

Acknowledgments

The lab

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Collaborators

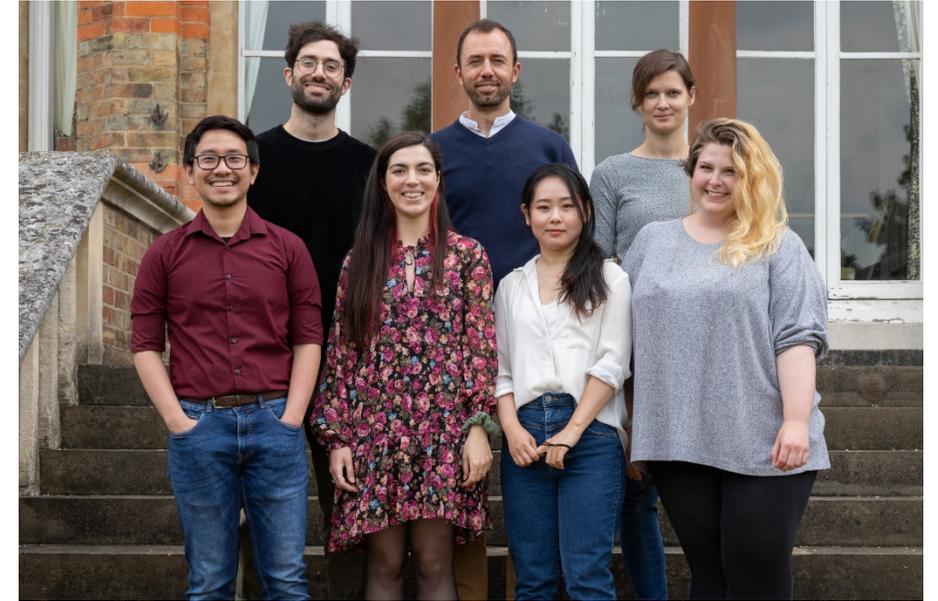
Mara Pennuto
Linda Greensmith
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Helen Miranda
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All patients, carriers and families

