# *Stronger Together: Tackling Rare Diseases Through Team Science*

November 5, 2025
Mini Med School 2025
Exploring Rare Diseases: Discovery to Treatment

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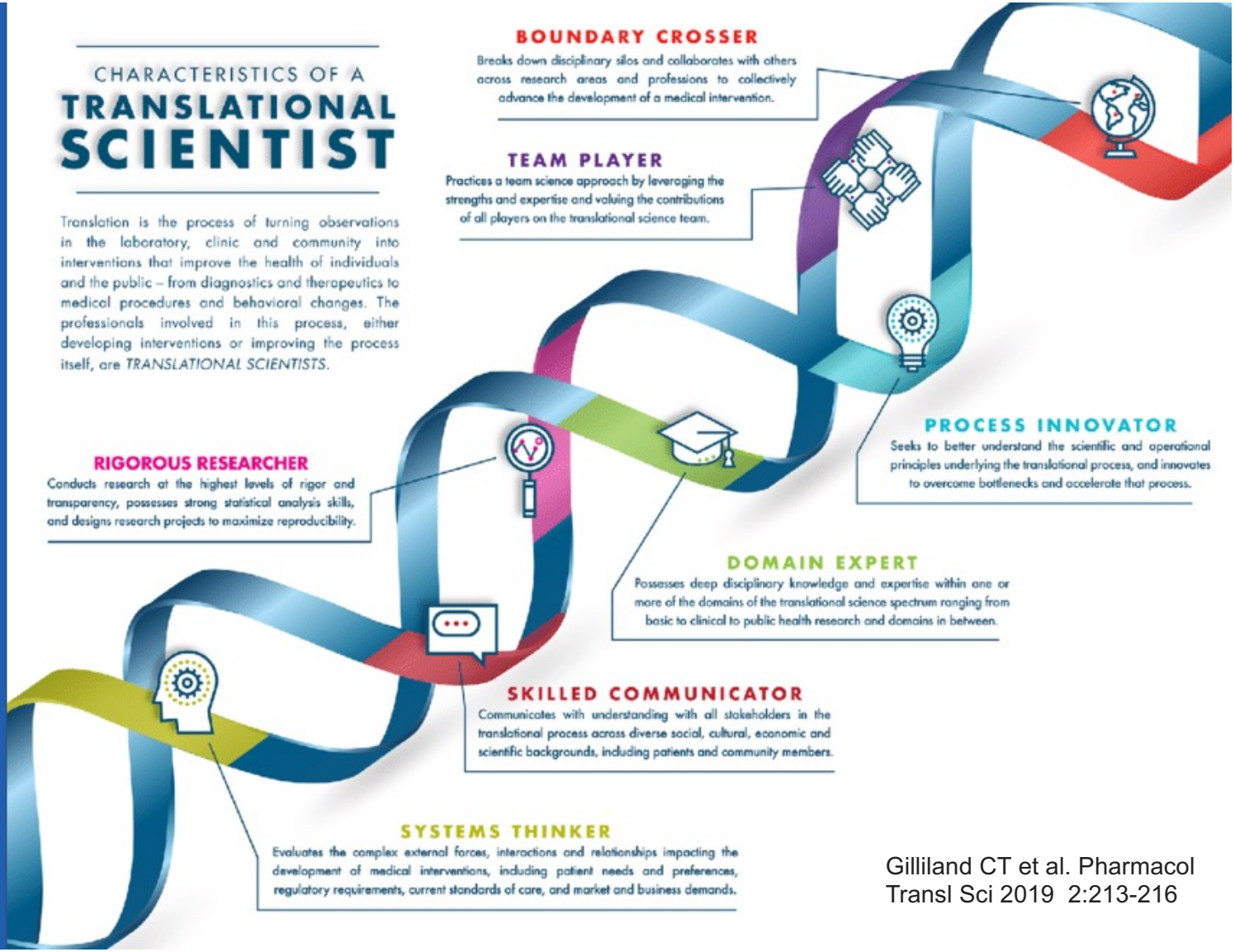
# Objectives of this presentation

- Introduce you to the family of **rheumatic diseases**
- Focus on childhood-onset **vasculitis** and the **Pediatric Vasculitis Initiative**
- Show how **collaboration can accelerate research** for an ultra rare disease
- Leave you **curious and inspired** about pediatric rheumatology

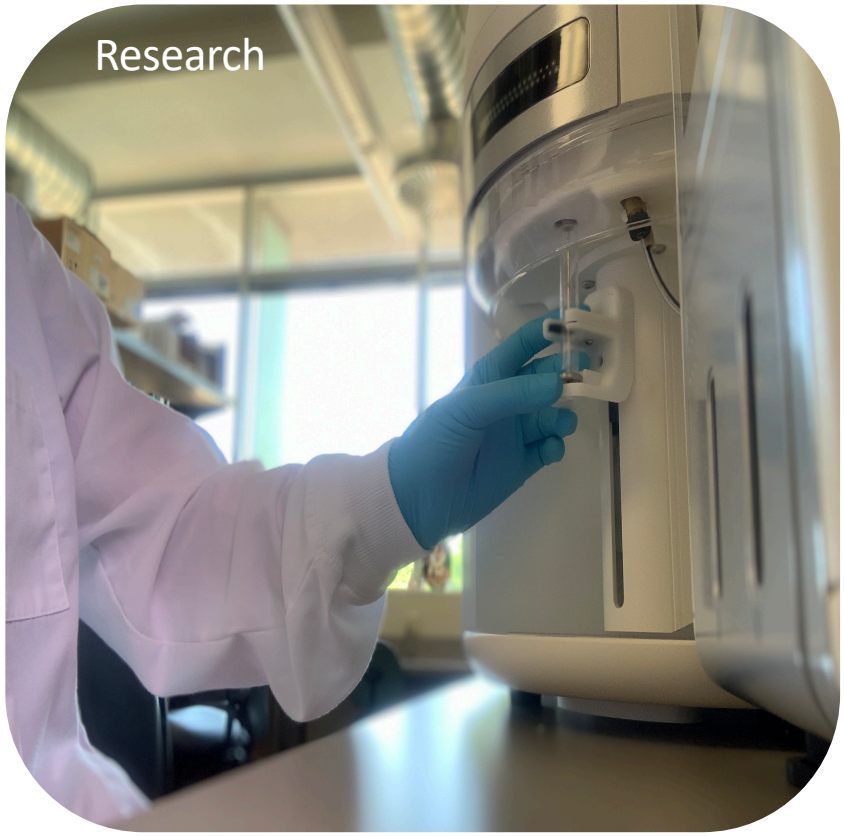
# From basic to translational scientist

Video from the NIH National Centre for Advancing Translational Sciences




Gilliland CT et al. Pharmacol Transl Sci 2019 2:213-216

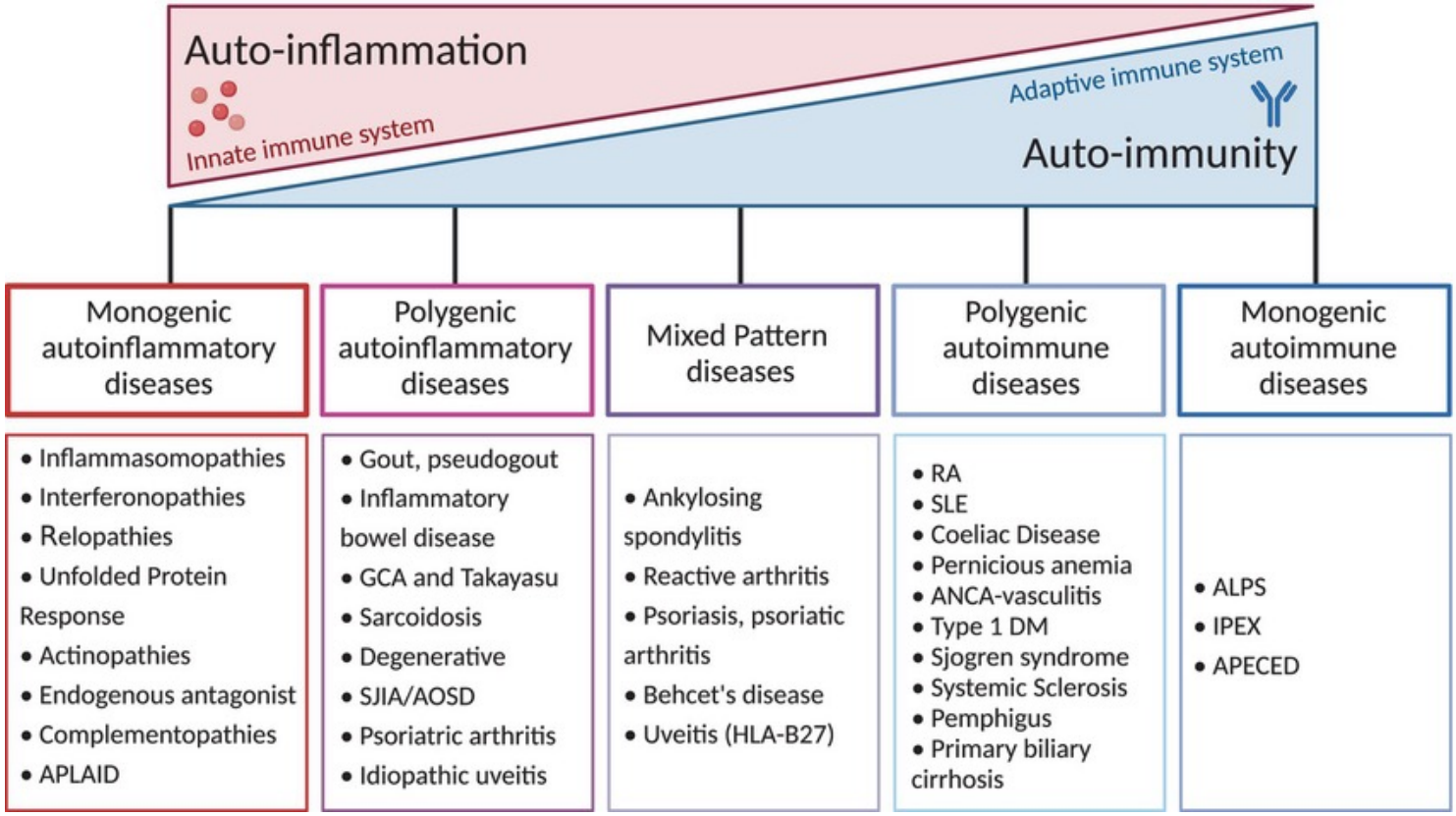
# BROWN LAB
TOGETHER IN DISCOVERY





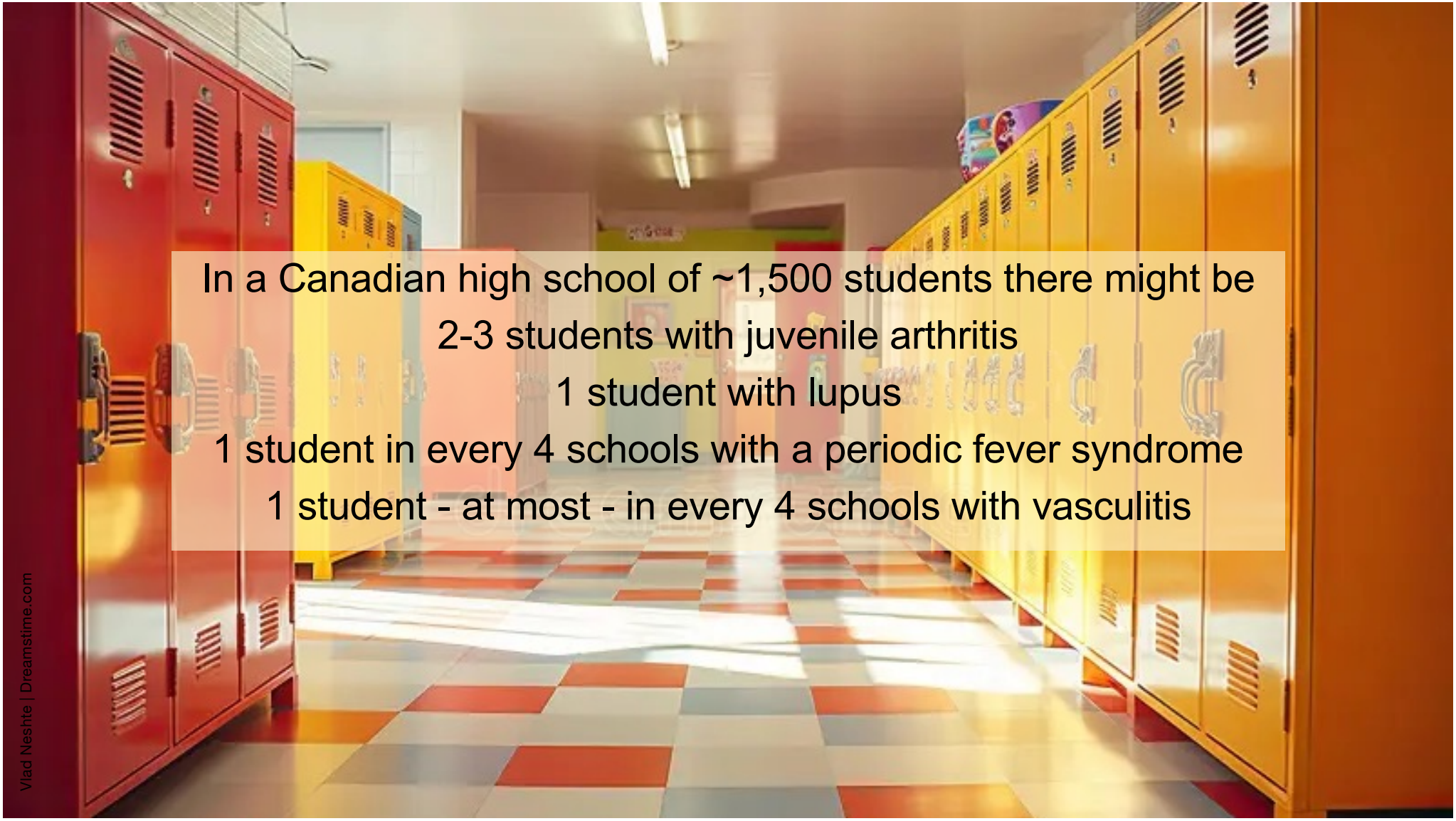

Learn more here

# What are Rheumatic Diseases?

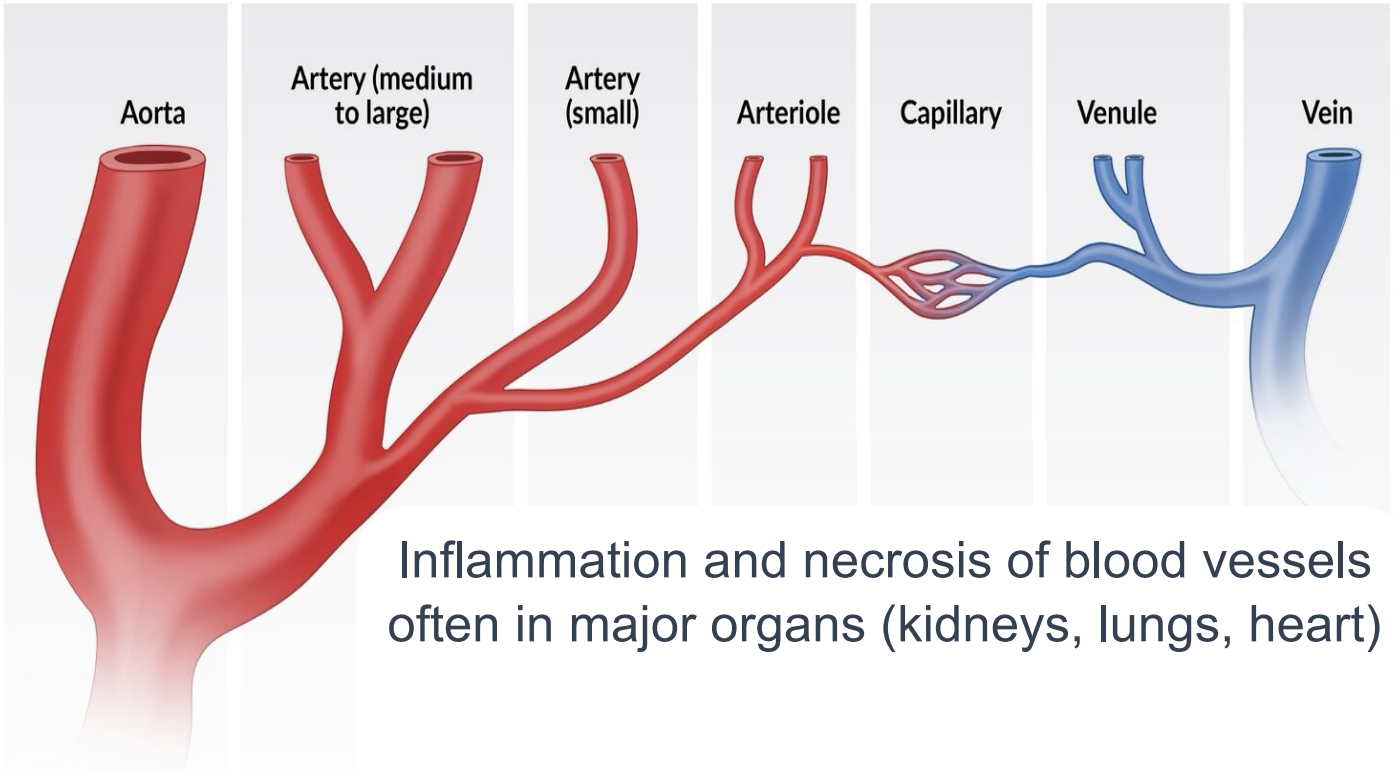



Merlo Pich LM et al. FEBS J. 2024 291(20):4414-4432.

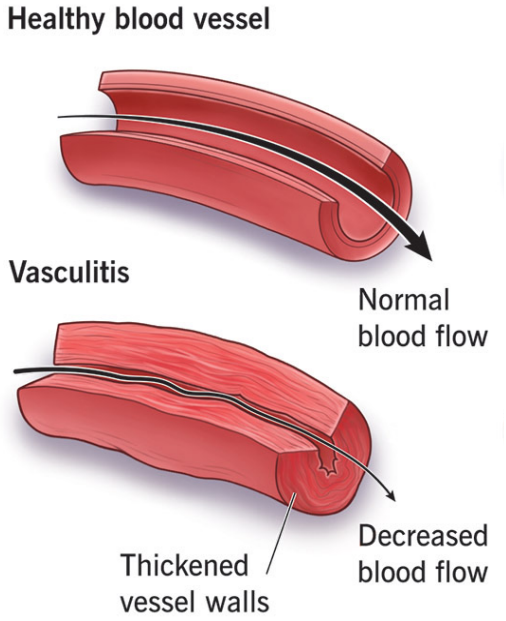
Rheumatic diseases have in common *uncontrolled inflammation* in joints, muscles, tissues, and major organs.



# What is Vasculitis?




Inflammation and necrosis of blood vessels often in major organs (kidneys, lungs, heart)




For a review of 2022 ACR/EULAR classification criteria, see Koster MJ and Warrington KJ Nat Rev Rheumatol 2022 18:309-310

Modified images from the Cleveland Clinic

# Pediatric vasculitis – the beginning

*1950s*

First reported case in a child.

*1970*

15 patients treated with cyclophosphamide.
12 (80%) achieved remission.

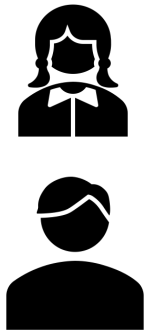
Fauci et al. Medicine 1973 52:533-561

*1990s*

150 patients (23 children) treated with cyclophosphamide.
89% achieved remission, but at a high cost with 86% experiencing morbidity.

Hoffan et al Ann Intern Med 1992 116:488-498.

# Childhood *vs* Adult onset Vasculitis

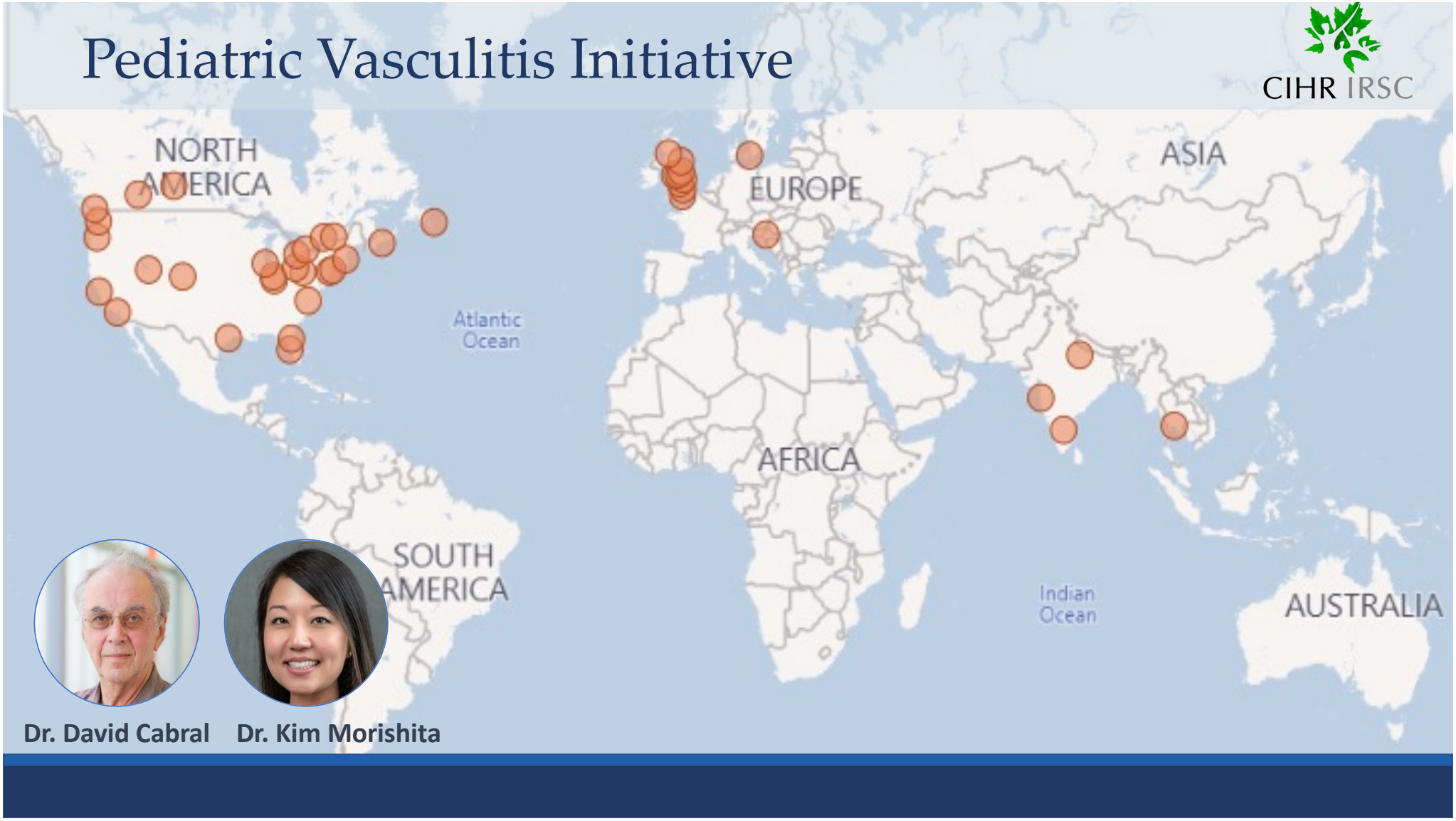


| | | |
|---|---|---|
| **10 – 14 yr** | **~80% female** | **0.2 - 6 / $10^6$** |
| **> 50 yr** | **~55-60% male** | **13 – 20 / $10^6$** |

Pediatric: Annual incidence from Geetha D et al Am J Kidney Dis 2020 75(1):124–37; **Sacri A-S** et al Nephrol Dial Transplant 2015 30(Suppl 1):i104-112, **Kouri AM** et al Pediatr Nephrol 2017 32(3):449-455, **Morishita KA** et al Arth Rheumatol 2017 69:1470-1479, **Calatroni M** et al Clin J Am Soc Nephrol 2021 16:1043–1051, **Yang J** et al Front Immunol 2022 13:857813, **Marlais M** et al Am J Kidney Dis 2023 18:119-122, **Tan L-W** et al World J Pediatrics 2024 20:506-516, **Toor KK** et al Arth Rheumatol 2025 77:606-614

# Pediatric Vasculitis Initiative

CIHR IRSC



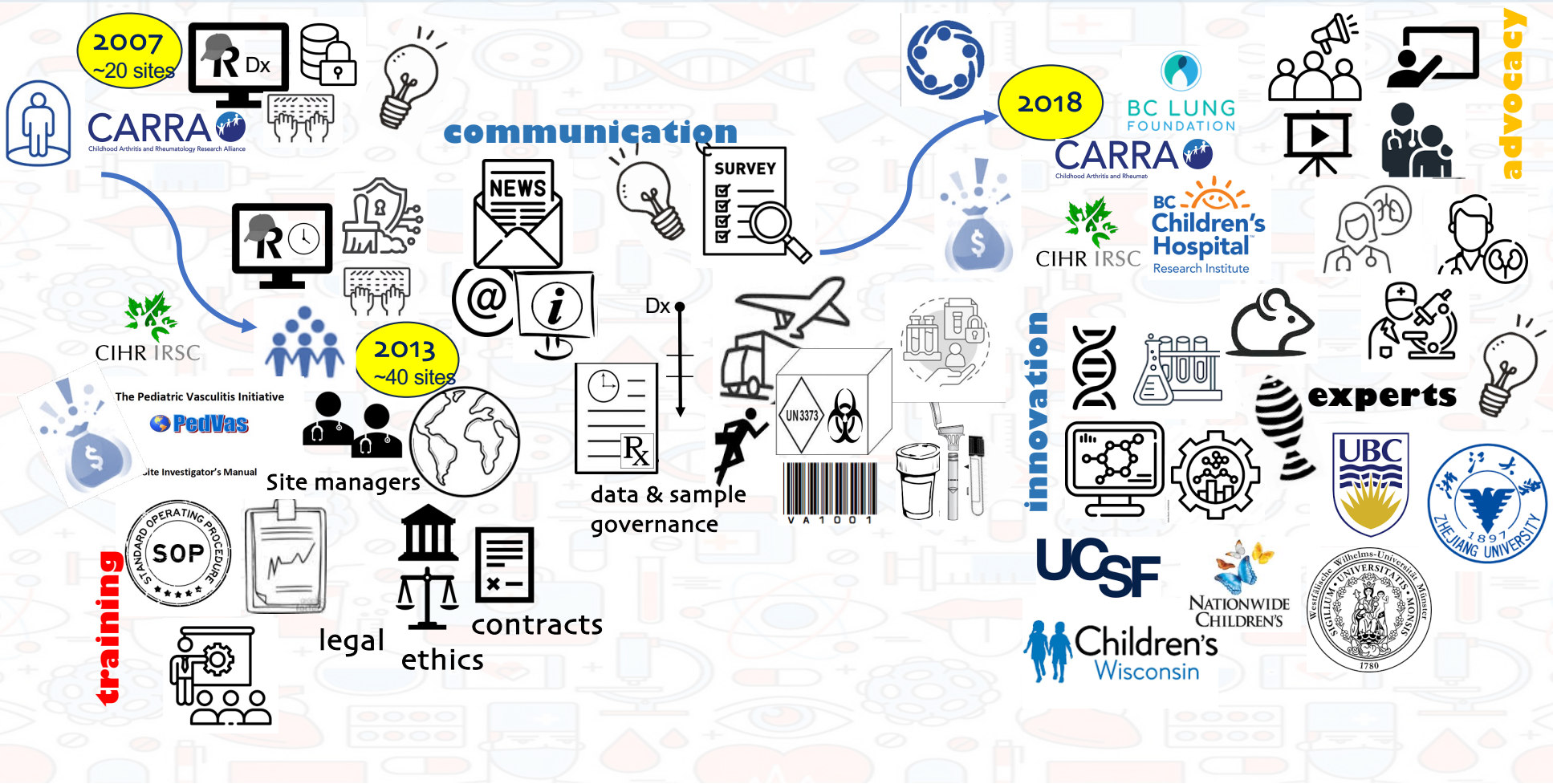



Dr. David Cabral



Dr. Kim Morishita

# Pediatric Vasculitis Initiative



# World's Largest Pediatric Vasculitis Resource

CIHR IRSC

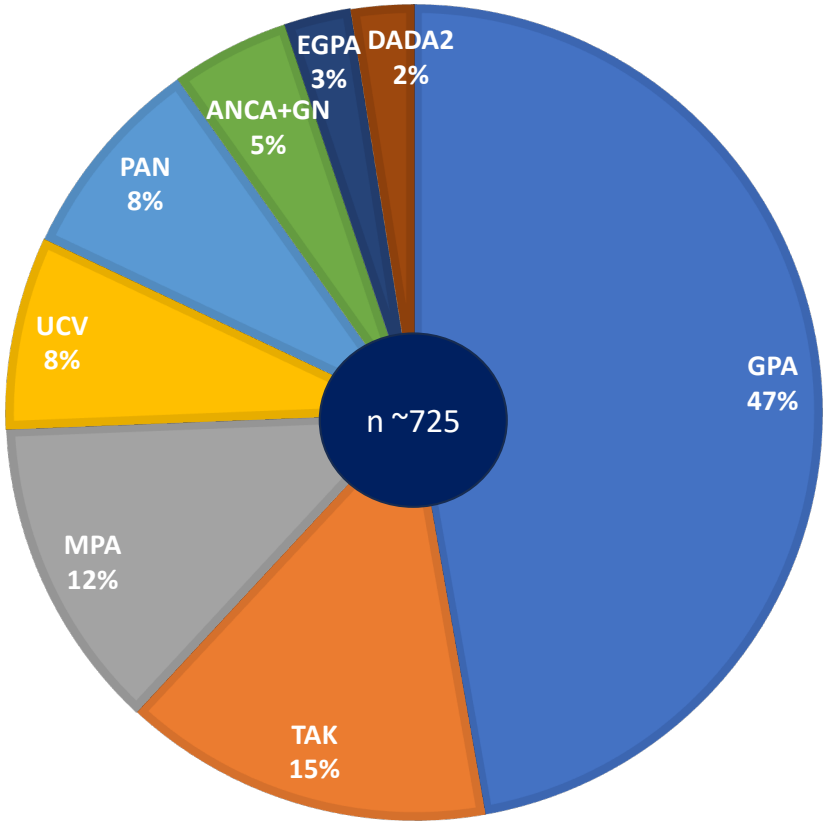




Image from BC Children's Hospital Biobank www.bcchr.ca/biobank