

Parent JOIN THE FIGHT.
Project END DUCHENNE.
Muscular
Dystrophy

 **Capricor**
Therapeutics™

HOPE-2 Open Label Extension
(1-Year Data Results)

Trial conducted by Capricor
National PI: Craig McDonald, M.D. (UC Davis)

June 27, 2022
NASDAQ: CAPR



Forward Looking Statements



Statements in this presentation regarding the efficacy, safety, and intended utilization of Capricor's product candidates; the initiation, conduct, size, timing and results of discovery efforts and clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including the ability to obtain regulatory approvals or otherwise bring products to market; the ability to achieve product milestones and to receive milestone payments from commercial partners; plans regarding current and future collaborative activities and the ownership of commercial rights; scope, duration, validity and enforceability of intellectual property rights; future royalty streams, revenue projections; expectations with respect to the expected use of proceeds from the recently completed offerings and the anticipated effects of the offerings, and any other statements about Capricor's management team's future expectations, beliefs, goals, plans or prospects constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Any statements that are not statements of historical fact (including statements containing the words "believes," "plans," "could," "anticipates," "expects," "estimates," "should," "target," "will," "would" and similar expressions) should also be considered to be forward-looking statements. There are a number of important factors that could cause actual results or events to differ materially from those indicated by such forward-looking statements. More information about these and other risks that may impact Capricor's business is set forth in Capricor's Annual Report on Form 10-K for the year ended December 31, 2021 as filed with the Securities and Exchange Commission on March 11, 2022 and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2022 as filed with the Securities and Exchange Commission on May 11, 2022. All forward-looking statements in this press release are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements.

CAP-1002 is an Investigational New Drug and is not approved for any indications. None of Capricor's exosome-based candidates have been approved for clinical investigation.

Call Participants

- **Linda Marban, Ph.D.** – Chief Executive Officer, Capricor Therapeutics, Inc.
- **Dan Paulson, M.D.** – Vice President of Clinical Development, Capricor Therapeutics, Inc.
- **AJ Bergmann, M.B.A.** – Chief Financial Officer, Capricor Therapeutics, Inc.
- **Craig McDonald, M.D.** – Professor and Chair of the Department of Physical Medicine and Rehabilitation and Director of the Neuromuscular Disease Clinics at the University of California, Davis. Dr. McDonald is an internationally recognized expert in the clinical management and rehabilitation of neuromuscular diseases including DMD. He is the national PI of Capricor’s HOPE-2 and HOPE-3 trials.
- **Suzanne Hendrix, Ph.D.** – CEO Pentara Statistical Group, Dr. Hendrix has been instrumental in analysis and reporting for multiple regulatory submissions and authored or co-authored over 150 peer-reviewed publications related to both clinical trial results and statistical approaches for clinical trials, most of which relate to analysis and design of trials for neurodegenerative diseases.

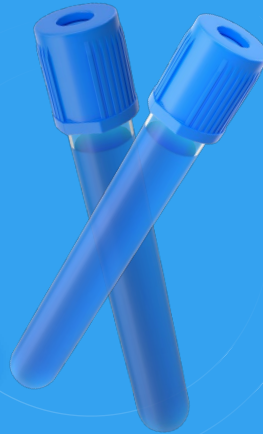
CAP-1002 Cell Therapy

● CAP-1002: Allogeneic Cardiosphere-Derived Cells (CDCs)

● Has been investigated in eight clinical trials

■ Clinical data demonstrating skeletal and cardiac improvements in DMD

● Sourced from transplant qualified hearts



● **Does not act by «stemness»**
the cells do not engraft into host tissue

■ **CAP-1002: Mechanism of action**

Cells secrete exosomes

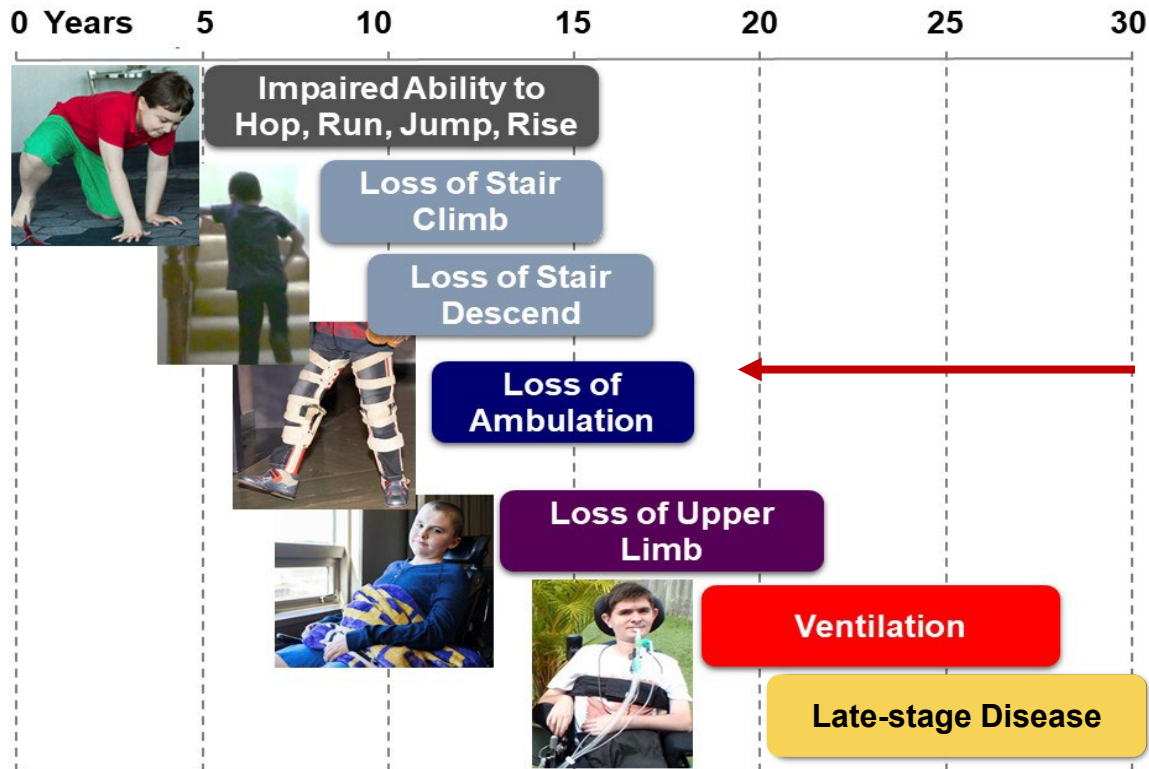
- Contain miRNAs, non-coding RNAs and proteins
- Trigger natural signaling with target cells
- Activate changes in cellular behavior

CAP-1002 Infusion Protocol is Easy

- I.V. (intravenous) administration every 3 months – ~45 minutes Procedure
- Simple oral premedication regimen before infusion
- Safety profile: no treatment related SAEs reported through 94 infusions in ongoing HOPE-2 open label extension
- CAP-1002 has been administered to over **200 subjects** to date across multiple clinical trials



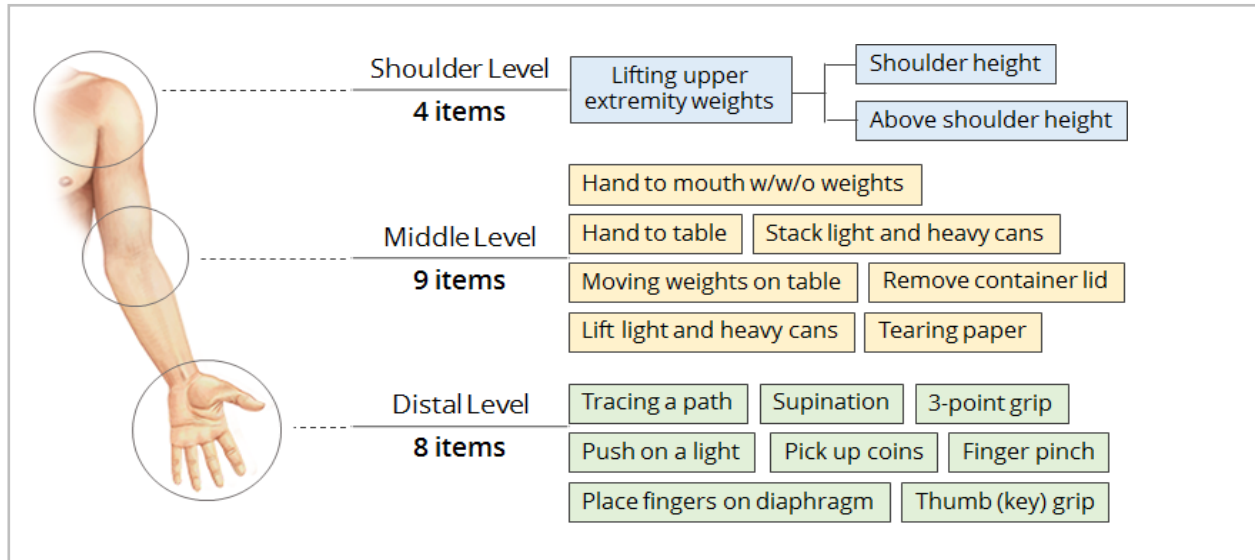
CAP-1002's Eligible DMD Population



CAP-1002's Eligible Patients

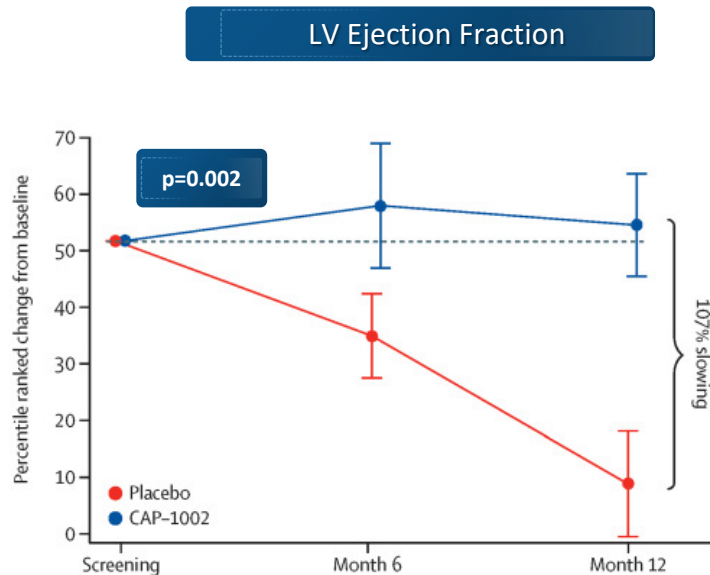
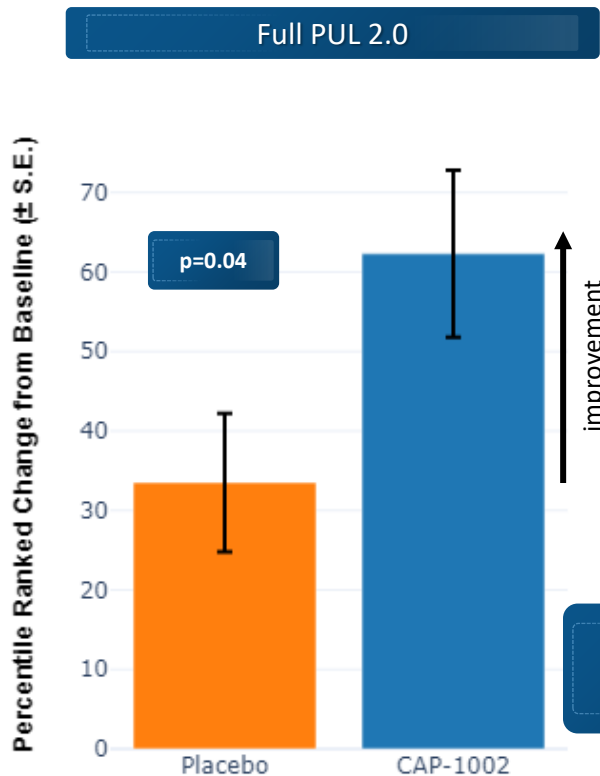
Estimated that over 50% of DMD patients in U.S. are non-ambulant

Measure: Performance of Upper Limb (PUL Test)



*Mayhew et al, 2019; Pane et al, 2018.

HOPE-2 Upper Limb and Cardiac Improvements



$\Delta=4\%$ in LVEF by cardiac MRI

Results published in [The Lancet](#), March 2022.

Capricor Therapeutics, Inc. | Developing Transformative Therapies from Bench to Bedside

Mixed Model for Repeated Measures (MMRM) analysis was performed using percentile ranked change from baseline as dependent variable and percentile ranked baseline score, treatment, visit, treatment-by-visit interaction, PUL entry-item score at randomization, and site as model effects. Adjusted model outcomes are report as least-squares means (LS-Mean).

HOPE-2 Open Label Extension Overview



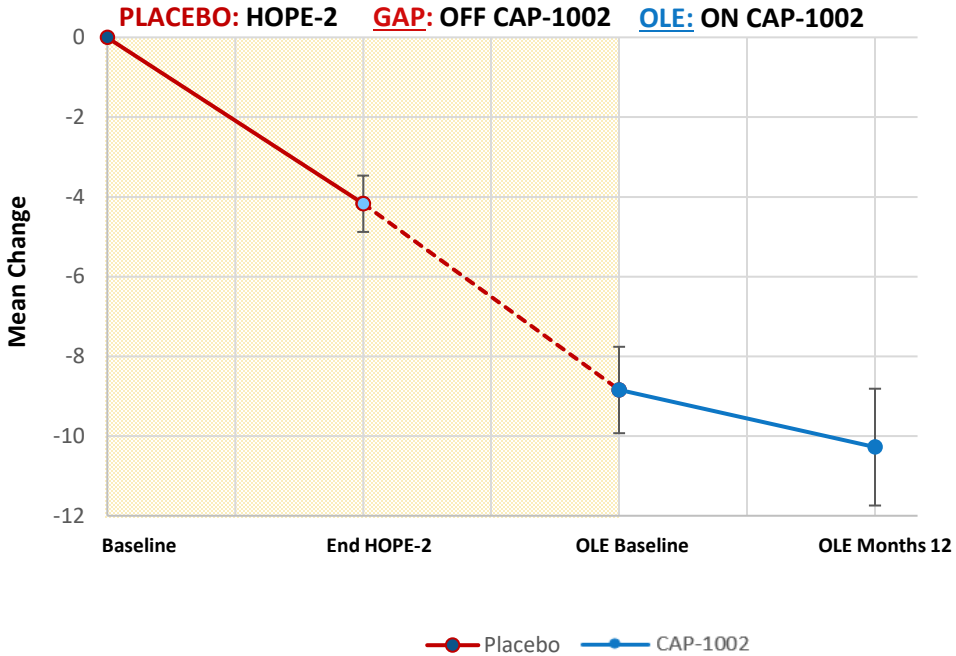
Open Label Extension Year 1 Overview

- n=13
- 6 original CAP-1002 patients
- 7 original placebo patients
- 1 patient withdrew consent

Patient Demographics

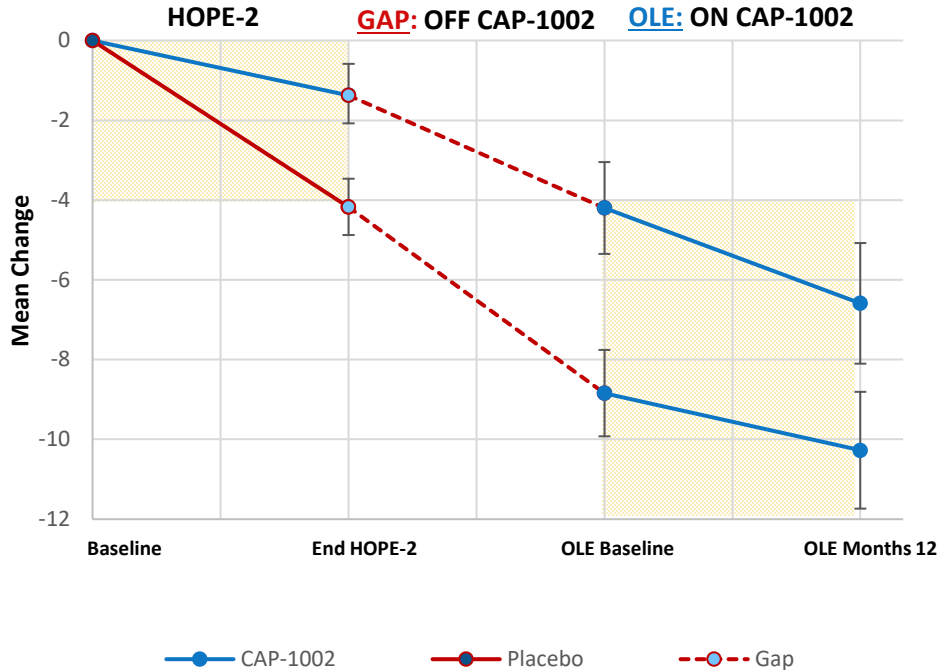
- Mean age: 13 years
- All patients were non-ambulant
- **All patients on stable regimen corticosteroids**

CAP-1002's Impact on Disease Progression



*One year change from baseline for a phase refers to a subject's change in one year during that phase.
*The linear mixed model uses all available data for all 20 subjects (12 completers).

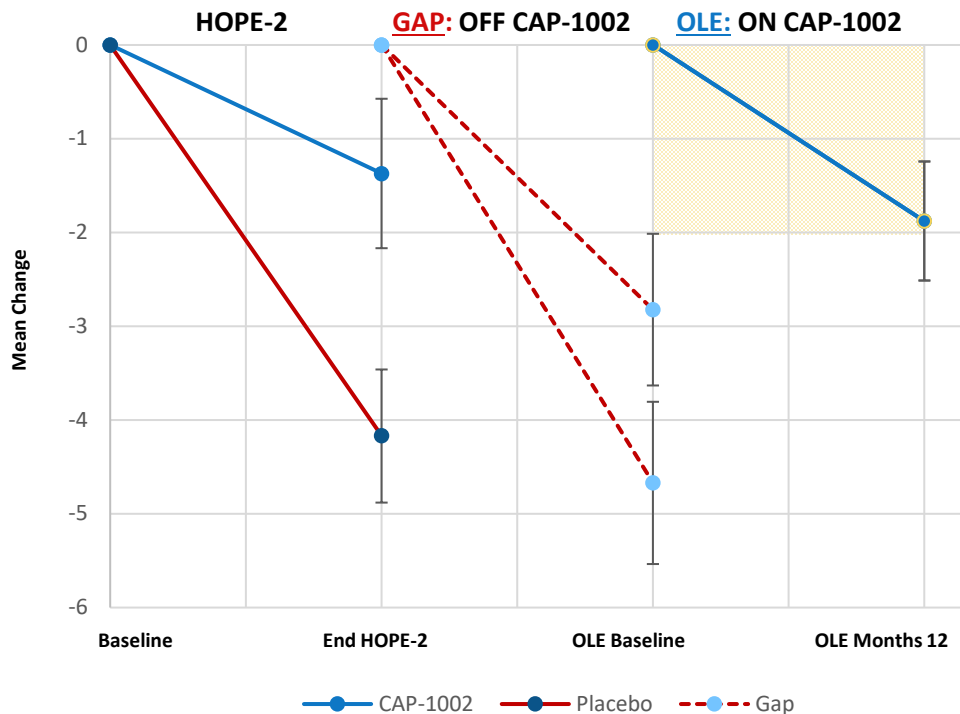
Impact of CAP-1002 on Rate of PUL Decline



*One year change from baseline for a phase refers to a subject's change in one year during that phase.

*The linear mixed model uses all available data for all 20 subjects (12 completers).

Accelerated Decline in PUL when Off-Treatment

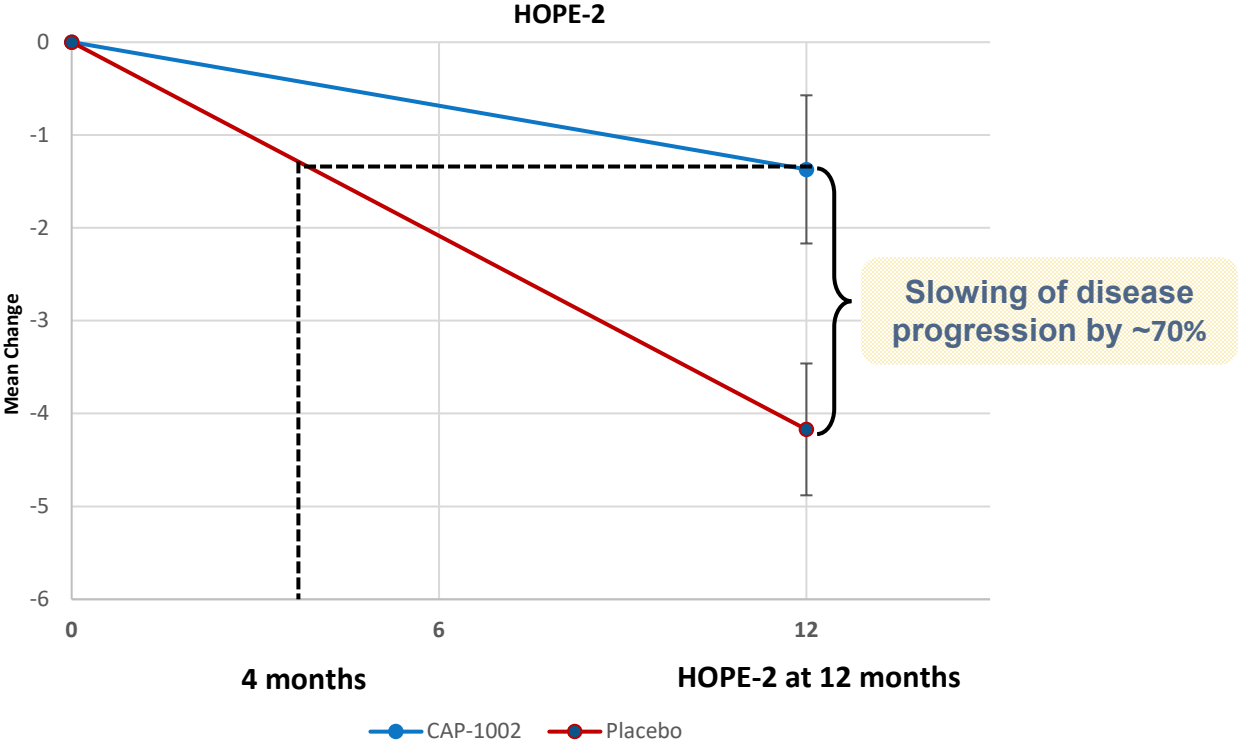


	Difference (PUL 2.0)	p-value
OLE vs. Placebo Group from HOPE-2	2.3	0.015
OLE vs. Placebo GAP	2.8	0.006

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*The linear mixed model uses all available data for all 20 subjects (12 completers).

CAP-1002's Impact on Disease Progression


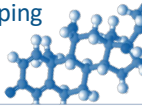











Results published in [The Lancet](#), March 2022.

Time is Muscle for DMD Patients

- **CAP-1002: Potential Disease Modifying Benefit**
 - Patients on therapy experience a slower progression of their disease
 - Urgency to initiate therapy: while treatment benefits are maintained (compared to placebo patients), loss of PUL points are never recovered
 - Potential sustained benefit: durable benefit of treatment at 2 years
- **Safety profile of CAP-1002 reinforced**
- **Preserved cardiac ejection fraction shown in HOPE-2**
- **HOPE-3 pivotal Phase 3 clinical trial open for enrollment**
 - Primary efficacy endpoint: PUL 2.0 at 12 months

CAP-1002 Opportunity for “Backbone Therapy”

 Options	Exon Skipping 	Gene therapy 	Steroids 	Other Therapeutics 
 Potential Synergy				
 We believe	<p>CAP-1002</p> <p>Potential for “backbone therapy” across non-ambulant population with safety demonstrated in over 200 patients</p>			

Acknowledgments

Patients and their families who participated in the HOPE-2 and HOPE-2 OLE Studies

- **Parent Project Muscular Dystrophy**
- **Craig McDonald, M.D. (UC Davis)**
- Coalition Duchenne
- CureDuchenne
- The Jett Foundation
- Action Duchenne
- MDA
- Casimir
- NS Pharma
- Capricor's DMD Advisory Board
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- Richard Finkel, M.D. (Nemours Children's Hospital)
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- Arun Varadhachary, M.D. (Washington University, Saint Louis Children's Hospital)
- Brenda Wong, M.D. (University of Massachusetts)
- Katherine Mathews, M.D. (University of Iowa, Children's Hospital)



Q&A