2019 Annual SMA Conference

SMA Clinical Care

Mary Schroth, MD
Chief Medical Officer, Cure SMA
June 29, 2019
Funding a Comprehensive Research Program: Over $80 Million in Total Research Funding

- **$9.1 Million** in clinical care funding over 3 years
  - Cure SMA Care Center Network (CCN)
  - Clinical Data Registry (CDR)
- Clinical Care Meeting

*Over $5M invested in care and research in both FY19 and FY20.*
Strategic Goals in Medicine and Research

• Build a comprehensive clinical care network and data registry to enable an optimal SMA standard of care

• Redefine the SMA disease classification
  – Clinical data registry
  – Patient reported data project

• Enable drug development through the industry collaboration

• Fund basic and translational research leading to combination therapies
SMA Clinical Data Registry

- All Care Center Network sites electronically send consented patient data from electronic medical record to the SMA Registry
  - 73 patients enrolled
  - 25% of all patients with SMA enrolled by 2021

Real World Evidence

- Improve the standard of care for all with SMA.
- Evaluate new therapy and combo therapy impact
- Provide evidence for payers
FDA Approved Treatments - Spinraza

- Approved 12/23/2016 for SMA all types and all ages
- Intrathecal administration
- Site locator: http://www.curesma.org/spinraza/sites/
FDA Approved Treatments - Zolgensma

- Approved 5/24/19 for SMA < 2 years of age, all types
- Intravenous administration
- Site locator: [http://www.curesma.org/zolgensma-administration-sites.html](http://www.curesma.org/zolgensma-administration-sites.html)

Mendell J et al, NEJM, 2017;377:1713
Zolgensma Trial vs Label vs Policy

-FDA label includes:
  • < 2 years of age at dosing
  • All copy numbers of SMN2
  • All types

-IV Clinical trials included:
  • < 9 months of age at dosing
  • Two copies of SMN2
Timing and Waiting

1. Evaluate and discuss therapies with your neurologist:
   - Safety and efficacy data
   - How given
   - Practical and process timing issues

2. Cure SMA recommends:
   - Don’t wait for a future treatment or clinical trial if other options are available now.

Time is neurons!

Early treatment → best outcomes
SMA and Care and Monitoring

- Continue close follow up by an experienced healthcare provider:
  - Monitor for symptoms of SMA
    * Continue care basics
  - Monitor for side effects of treatments
  - Optimize the benefits of treatment
  - Discuss new information about SMA
Cure SMA Medical Advisory Council

• See program for MAC membership list
• Clinical Care Meeting
  – Over 350 clinicians from around the world
    • Networking
    • SMA Care Discussions
• Medical topic workshops
• Summit of Strength speakers
Summit of Strength Program

Local one day program for people of all ages and types of SMA, and their loved ones and caregivers

This is a free program which includes breakfast, lunch and parking for all attendees.

The dates and cities for the 2019 Summit of Strength Program are:

March 2 – Dallas, TX
March 9 – Fort Lauderdale, FL
March 30 – Seattle, WA
May 4 – Grand Rapids, MI
May 11 – Milwaukee, WI
June 1 – Kansas City, MO
July 20 – Denver, CO
July 27 – Salt Lake City, UT
August 3 – Long Island, NY
August 10 – Chicago, IL

August 24 – Cincinnati, OH
August 31 – Anaheim, CA
September 7 – Philadelphia, PA
September 14 – Rochester, NY
September 21 – Minneapolis, MN
September 28 – Newark, NJ
October 5 – San Ramon, CA
October 12 – Boston, MA
October 19 – Charleston, SC
November 9 – Orlando, FL

Registration is open – Visit the Cure SMA Events Calendar at http://www.curesma.org/get-involved/event-calendar/ to register for your local Summit of Strength today!
Cure SMA Guide – Mobile Device App

• A family support program
• Provides readily available important information for SMA care
• Use at home and on-the-go.

• Contains all SMA Care Series booklets
2019 Annual SMA Conference

• Please ask questions
  – This conference is for you
  – Sessions are organized by topic
• Please provide feedback
  – Complete evaluations
• Many medical healthcare providers at this conference
  – The best way we can help you with your care locally:
    • Take back the information presented
    • Slides will be available on line from those presenters with slides
    • Ask your local care provider to contact us
      – Patientcare@curesma.org
Conference Workshop Schedule and Room Changes - Aquatics

Saturday

Aquatic Physical Therapy for Fun & Function, Teens and Adults
New time: 11:15am – 12:30pm

Aquatic Therapy Questions & Answers
New time: 2:00pm – 3:30pm
New room: Center Ballroom

Sunday

Aquatic Physical Therapy for Fun & Function, Children with Independent Head Control
New time: 8:45am – 10:15am

Aquatic Physical Therapy for Fun & Function, Children with Supported Head Control
No change: 10:30am – 12:00pm
Conference Workshop
Schedule and Room Changes - Breathing

Saturday
I Need to Cough, Ways to Clear Your Lungs
New time: 11:15am – 12:30pm
New room: South Ballroom A

Sunday
Breathing Strong All Day Long
New time: 8:45am – 10:15am
New room: South Ballroom B
2019 New Workshops Saturday

• Workshop #1, 11:15am-12:30pm
  – **Power Soccer**…More than just a Sport!
• Workshop #2, 2:00-3:30pm
  – Supporting the **Emotional Health** of SMA Affected Family Members
  – Relevant **Assistive Technology Tools** for Individuals with SMA
  – Spouses and Significant Others Sharing Your Experience in Nile *(changed time and room)*
• Workshop #3, 3:45-5:15pm
  – **Mom’s Time**: A Workshop for Mothers Only

• **Evening Special Events:**
  – Family Friendly Poster Session 6:30-8:30 pm
  – PJ Party and Movie Night 7:30-9:30 pm
  – Adults with SMA Reception 8-10 pm
2019 New Workshops Sunday

- Workshop #5, 10:30am-12:00pm
  - A Parent’s Guide: How to Change your Child’s Behavior
- Workshop #6, 1:30-3:00 pm
  - Unpacking SMA Clinical Trials
  - Young Adults Talk it Out (Ages 18+)
- Workshop #7, 3:15-4:30pm
  - Managing Stress in a World That Isn’t Always Accessible
  - Rehabilitation care considerations in the new era of SMA
- Evening Special Events:
  - Cure SMA Evening at the PARK 4:30 pm
2019 Conference Highlights

• **Family Breakfast Symposium** Sponsored by Genentech
  – Saturday, 7:15 am – 8:30 am in South Exhibit Hall

• **Family Lunch Symposium** Sponsored by Biogen
  – Saturday, 12:30 pm – 1:45 pm in South Exhibit Hall

• **Family Lunch Symposium** Sponsored by AveXis
  – Sunday, 12:00 pm – 1:15 pm in South Exhibit Hall
2019 Conference Highlights Monday

- It’s a Wonderful Life/Closing General Session
  - 9:00 am - 11:15 am, Center Ballroom
HYDRATE and SUN PROTECTION

• Drink lots of water

• If tube feeding dependent:
  – Increase fluids by 10-20%
    • Prefer electrolyte solutions
      – Pedialyte or Enterolyte
      – Gatorade or juice

• With sun exposure:
  – Use Sun screen
  – Wear protective clothing to cover
  – Wear sunglasses
Vendors

- 16 Exhibitors
- Open all day Saturday and Sunday
Conference Feedback – Paper or Online Survey

• Tell us:
  – What worked and What didn’t.
  – What was missing.
  – Brand new ideas!

FILL OUT THE CONFERENCE SURVEY
FOR YOUR CHANCE TO WIN AN APPLE WATCH

The success of our conference can only be improved by the feedback that we received from you! Please take the time to provide any comments by filling out our conference survey, and the workshop surveys. You can find hard copies of your surveys in your conference folder. Please drop your completed surveys into the many bins throughout the meeting space, at your workshops or at the Registration Area.

All participants who complete the surveys by 10:30am on Monday, July 1st, will have their name entered into a raffle.

The winners of the raffle will receive a brand new Apple Watch!

You can also fill out the survey on-line at http://events.curesma.org/2019annualsmaconference
Thank you!