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FARA Friedreich's Ataxia Research Alliance

How does the Debt Ceiling debate impact FA research? A Call to Action!

President Biden and Speaker McCarthy just announced they have reached an agreement regarding the debt ceiling. What exactly is the debt ceiling? The debt ceiling, also known as a debt limit, is the maximum amount of money that the US government is authorized to borrow to fulfill its financial obligations. When the federal government spends more than it brings in through taxes and other revenue, it must borrow money to pay its bills.

The Constitution requires Congress to authorize borrowing. A debt limit was first created in 1917 to finance mobilization efforts in World War I and further codified in 1939 to allow the Treasury to issue debt up to a certain limit without approval from Congress. The debt ceiling has been raised 78 times since 1960, under both Republican and Democratic Presidents. Raising the debt limit itself does not authorize new spending; it allows the US government to pay for decisions already legislated for in prior budgets. If the debt limit is not raised by June 5, the US government will be unable to pay its bills and will default.

Congress is also in the process of negotiating next year's budget, fiscal year 2024, which will cover Oct 1, 2023 - Sep 30, 2024. The current debate on raising the debt limit rests on an effort to secure an agreement to cut certain discretionary spending in future budgets.

The deal reached last night would raise the debt limit for two years in exchange for a cap on certain non-defense discretionary spending. Included in those provisions is lifesaving medical research and R&D programs that keep the US a world leader in innovation. Our partner Research!America shared the following:

- Research!America Board Member Kafui Dzirasa, MD, PhD, makes a compelling case for why the "U.S. Must Invest in Emerging Scientists" in Inside Higher Ed. He proposes raising salaries for NIH-funded researchers seeking to establish themselves as a critical part of the R&D pipeline in academia. As Dr. Dzirasa points out, ensuring these researchers remain in academia is essential to prevent the further erosion of our capacity to compete as the world leader in science and technology. (STAC shows how we are losing ground.)

Instead, the proposed agreement will keep these programs basically flat in FY24 and be limited to a 1% increase in FY25. The impact for FY24 will be buffered somewhat by money that can be clawed back from unspent funds from other programs, an option that will not be available for FY25.

The loss of robust funding to federal research agencies like the NIH and FDA in the next two fiscal years would directly impact FA research. Both agencies are instrumental in fostering exciting new medical advancements and are in need of more, not less, financial support to enable them to have the resources required to expedite lifesaving treatments to FA patients.

The proposed agreement needs to be voted on by Congress within the next few days. Legislators need to hear your story, your voice! The time to act is now.

- **Call or email your Members and tell them how much your family is depending on advancing medical research. Ask them to prioritize federal research budgets so that agencies like the NIH and FDA can continue to expedite FA treatments. You can find your Senator here and your House Member here.**
- **Post on social media and tag your Members. Make sure to add #DontCutCures #CureFA**

Capitol Hill Updates

MVP Act Reintroduced

In April, the Medicaid Value Based Payments for Patients (MVP) Act was reintroduced to the House as H.R. 2666. This bipartisan legislation is led by Rep. Brett Guthrie (R-KY-2), Anna Eshoo (D-CA-16), and four other co-sponsors. The bill would allow "for the use of varying best price points under value-based purchasing arrangements for purposes of the Medicaid Drug Rebate Program." It would also require the GAO to study the impact of value-based purchasing on federal healthcare programs.

FARA joined 130+ advocates for the Alliance for Regenerative Medicine Congressional Fly-In on May 10 to support this initiative.

RARE Act Reintroduced

Senator Tammy Baldwin (D-WI) reintroduced the Retaining Access and Restoring Exclusivity (RARE) Act, S.1214. This bill was previously introduced by Senator Baldwin in the 117th Congress. The RARE Act would codify how FDA currently interprets the Orphan Drug Act's exclusivity provisions, limiting the seven-year exclusivity period for orphan products to the approved use of indication, rather than the entire disease or condition as the 11th Circuit held in Catalyst Pharms., Inc. v. Becerra. The RARE Act would incentivize companies to continue investing in therapeutics across the disease population, rather than targeting the narrowest indication for approval.

FARA joined 78 organizations in a letter to Congressional Members in support of the RARE Act. Click here to read the letter.

UPDATE: On May 11, the Senate HELP Committee voted 21-0 in support of the RARE Act. The bipartisan and unanimous vote for this important legislation would not have been possible without the strong support of our rare community!

Congressional Letter Urges the FDA to Improve Reliability in Reviewing Applications for Rare Disease Therapies

Senators Amy Klobuchar and Roger Wicker (R-MS) and U.S. Representatives Gus Bilirakis (R-FL) and Doris Matsui (D-CA), co-chairs of the bipartisan bicameral Rare Disease Congressional Caucus, led a group of 25 members of Congress in a letter to Commissioner Robert Califf. In the letter, members recognized the progress made since the adoption of the Orphan Drug Act but highlighted that most rare diseases still lack a treatment. The FDA was asked to bring more reliability and consistency to the process of reviewing rare disease therapies.

Learn more: here.

President Biden nominates cancer surgeon Monica Bertagnolli to head NIH

On May 15, 2023, President Biden nominated cancer surgeon Monica Bertagnolli to be the next director of the National Institutes of Health, a position that has been vacant for over a year. If confirmed, she would make history as the second woman to hold the permanent director position at the NIH. Currently serving as the director of the National Cancer Institute, Bertagnolli has played a significant role in advancing President Biden's Cancer Moonshot initiative. Her firsthand experience as a breast cancer patient brings a valuable perspective to the position.

Upcoming Advocacy Events

Calling all youth and teen advocates!

Deadline to Register is May 31

Rare Disease Legislative Advocates (RDLA) invite members of the rare disease community between 10 and 18 years old to participate in their first Virtual Youth & Teen Hill Advocacy Day. **Registration closes May 31st, 2023.**

Anyone between the ages of 10 to 18 with a connection to the rare disease community is welcome to participate, as well as their parents or guardians.

Advocates will have the opportunity to virtually meet with their Members of Congress and share their rare disease story. Prior to meetings with Members of Congress, advocates will have virtual trainings on how Congress creates laws, how to communicate with policymakers, and how to understand key policies affecting the rare disease community, designed for youth and teens.

When?

Virtual meetings with Members of Congress will take place on Thursday, June 22, 2023 between 9 am and 5 pm ET.

Dates for the required virtual trainings are:

- Sunday, June 4th, 4:00 pm ET: Virtual Meet and Greet
- Thursday, June 8th, 5:00 pm ET: General Training Webinar
- Thursday, June 15th, 5:00 pm ET: Share Your Story with Policymakers Webinar
- Friday, June 23rd, 3:00 pm ET: Virtual Celebration Meet-Up

Recordings will be made available to advocates who cannot attend webinars in live time.

Register Here

Rare Across America Registration is Now Open

Rare Disease Legislative Advocates (RDLA) organizes meetings with your Members of Congress and/or the Member's staff during the congressional August recess. Virtual and In-Person meetings will take place between August 7th and 18th. All House meetings will be held in-person at the Representative's district office, and all Senate meetings will be virtual. **Registration closes July 11th - Register here.**

The RDLA team also helps you prepare for meetings, provides legislative resource materials, and hosts pre-meeting training webinars. No prior experience is necessary but you need to register for the trainings separately.

Webinar Trainings: To register, click here.

July 18th, 2:00 pm ET: Rare Across America General Training Webinar: This webinar will cover everything you need to know for Rare Across America including

summaries on the official asks, what to expect during your meetings, what you should do to prepare for your meetings, how to follow up after your meetings and more!

July 25th, 2:00 pm ET: Share Your Story with Policymakers Webinar: This webinar is an opportunity to practice sharing your rare disease story! You will have the chance to practice your "pitch" and receive feedback from coaches.

July 27th, 2:00pm ET: Team Coordinator Training Webinar (No registration required, attendance information will be sent to all confirmed Team Coordinators)

Announcements

Research!America Shares Report Highlighting the Return on Investment of R&D

The value of federal investments in R&D is illustrated vividly in a new report released by the Science & Technology Action Committee (STAC) on "The Incredible, Incomparable ROI of R&D." The report is essential – both to read and to share. It succinctly describes the urgency of the present moment: today, the U.S. government spends an amount equal to only 0.7% of the nation's GDP on R&D, down from 1.9% in the 1960s – an era that drove decades of scientific progress and prosperity and made the U.S. the unrivaled world leader in R&D for half a century.

FDA/CDER and Johns Hopkins Webinar on Clinical Trials for Rare Diseases

On Tuesday and Wednesday, May 2–3, 2023, the FDA/CDER and Johns Hopkins University cohosted **a virtual workshop** titled, "Addressing Challenges in the Design and Analysis of Rare Disease Clinical Trials: Considerations and Tools Virtual Workshop."

Topics included the use of natural history studies and registry data to inform rare disease drug development, as well as tools and approaches to design, conduct, and analyze trials for rare diseases.

Watch the recording here.

State Update on Newborn Screening

Louisiana HB 200

HB 200 would require the Louisiana Department of Health to annually consider whether to recommend the inclusion of new RUSP conditions to the state's screening program. It would also require that if the department does not adopt a condition for screening within three years of its addition to the RUSP, it must report to the advisory committee every six months following on the reasons for the non-adoption. HB 200 passed in the House and is under consideration in the Senate.

To see the status of other newborn screening bills across the states, click here.