ISSUE BRIEF: ABLE Improvement Bills

Legislative Request

Please cosponsor three Achieving a Better Life Experience (ABLE) improvement bills that will soon be introduced in the House and Senate: the ABLE to Work Act, the ABLE Financial Planning Act and the ABLE Age Adjustment Act.

D **Senate:** Senate offices may cosponsor by contacting Natasha Hickman with Senator Richard Burr at Natasha_Hickman@burr.senate.gov or (202) 224-3154 or Livia Shmavonian with Senator Bob Casey at Livia_Shmavonian@casey.senate.gov or (202) 224-6324.

D **House:** House offices may cosponsor by contacting Megan Perez with Rep. Cathy McMorris Rodgers at Megan.Perez@mail.house.gov or (202) 225-5107 or Jacqueline Usyk with Rep. Tony Cardenas at Jacqueline.Usyk@mail.house.gov or (202) 225-6131.

Background and Summary

The Stephen Beck Jr. Achieving a Better Life Experience (ABLE) Act (Public Law 113-295) was signed into law on December 19, 2014. The ABLE Act amends the Internal Revenue Service Code of 1986 to allow for the establishment of Section 529A tax-free savings accounts for individuals with disabilities. The funds in the ABLE account do not count toward the $2,000 cap on assets that is required to remain eligible for critical government supports. An ABLE account may fund a variety of essential expenses for individuals with disabilities including medical and dental care, education, community based supports, employment training, assistive technology, housing and transportation. The federal ABLE Act authorizes the states to develop their own ABLE programs, and many states have moved quickly to pass ABLE laws and are in various stages of developing their ABLE programs. As of March 28, 2017, 18 states have launched ABLE programs.

Three bills will soon be introduced in the House and Senate to enhance the ABLE Act. Each one of the three ABLE improvement bills would increase the breadth and reach of ABLE accounts. Collectively, these measures represent the commitment of the original sponsors of the ABLE Act to make adjustments over time to reflect some of the provisions that were not included in the final version of that law. Specifically:

- **The ABLE to Work Act** would allow ABLE beneficiaries who work and earn income, but do not participate in an employer's retirement plan, to save additional amounts in their 529A (ABLE) account up to the federal poverty level (currently $12,060) in addition to the $14,000 annual maximum contribution. Beneficiaries would also be eligible for the Saver's Credit, an existing federal tax credit that low and middle-income individuals can currently claim when they make contributions to a retirement account.

- **The ABLE Financial Planning Act** would allow ABLE beneficiaries to roll over regular 529 accounts to 529A (ABLE) accounts up to the annual maximum contribution, and also would allow for a reverse-rollover if the beneficiary ceases to be disabled. This bill would be particularly helpful for families who set up 529 accounts before receiving a child's diagnosis, or for teenagers who incur life-changing events that render them unable to go to college and use their 529 funds for their original purpose.

- **The ABLE Age Adjustment Act** would raise the age of onset of disability from 26 to 46, which is halfway to retirement age. This would allow more individuals who become disabled later in life to take advantage of the benefits of ABLE accounts.

Key Messages

Incentivizing Employment - Many people with disabilities are reluctant to take a paid employment position or have declined raises or extra hours because it could jeopardize their Medicaid and SSI benefits. The ABLE Act's $14,000 annual contribution cap does little to incentivize employment since it is the aggregate of all contributions to the ABLE account (including earned income), and employed beneficiaries are still unable to contribute to employer-provided retirement accounts, such as 401(k) plans. The *ABLE to Work Act* would bring more people with disabilities out of poverty and incentivize them to work by permitting them to save their earnings in an ABLE account. Beneficiaries will be able to weigh the additional income they can earn and save against their monthly Social Security check, which may decrease as income rises.

Protecting Against Life-Changing Events - Many families set up 529 accounts before receiving a child's diagnosis, or have teenagers who incur life-changing events that render them unable to go to college and use their 529 funds for their original purpose. The *ABLE Financial Planning Act* would allow such families to roll over regular 529 accounts to 529A (ABLE) accounts up to the annual maximum contribution, and will also allow for a reverse-rollover if the beneficiary ceases to be disabled.

Treating People with Disabilities Equitably - Currently, individuals with a severe disability that occurred prior to the age of 26 are eligible to open an ABLE account. Many debilitating diseases and conditions can occur later in life, including multiple sclerosis, Lou Gehrig's disease or paralysis due to an accident. The *ABLE Age Adjustment Act* would increase the age of onset of disability to 46 to allow more individuals to save money to help cover the costs of short, medium and long-term disability related expenses.

Answers to Questions about the ABLE Improvement Bills

1. **Which of the three ABLE improvement bills is most important to people with Down syndrome?**

NDSS supports all three bills. However, our top priority is the *ABLE to Work Act* because we believe it will have the most impact on the Down syndrome community, and it will advance our employment campaign, #DSWORKS®. Since Down syndrome is diagnosed at or before birth and is a lifelong condition, the other two bills are not as applicable to our population. NDSS will be advocating for all three bills on behalf of our cross-disability partners who have designated them as priorities, but the *ABLE to Work Act* is our priority.

2. **Why is the increased contribution limit in the ABLE to Work Act tied to the federal poverty level?**

The federal poverty level is a readily available number that is regularly adjusted by the US Department of Health & Human Services. Since so many individuals with disabilities are impoverished, the need for people with disabilities to save up to the poverty line is very compelling. Also, this cap ensures that the impact in terms of lost revenue to the government is de minimus, while also ensuring that disabled individuals with high-paying jobs will not be able to use ABLE accounts as a tax shelter.

3. **If the three ABLE improvement bills are enacted, what is the estimated cost to the government in terms of increased spending and/or lost revenue?**

In the last Congress, two of the bills-the ABLE to Work Act and the ABLE Financial Planning Act-advanced through the Senate Finance Committee and were scored as "negligible," thereby not requiring an offset. By

contrast, it was determined that the ABLE Age Adjustment Act, which increases the age of onset of disability from 26 to 46, would cost $2 billion over ten years.

4. **Why was the age limit in the original ABLE Act set at 26, and the age limit in the ABLE Age Adjustment Actset at 46?**

As introduced, the original ABLE Act did not have any age restriction. After review by the Congressional Budget Office (CBO) and the Joint Committee on Taxation (JCT), the cost associated with enactment of the original ABLE Act was prohibitive. According to CBO, at least 75 percent of the costs were associated with the lack of an age restriction. By limiting eligibility to age 26, the sponsors dramatically reduced the cost, which facilitated enactment of the bill. At the time, the bill sponsors vowed to work address this inequity, and the new proposed age limit of 46, while still inequitable, is an effort to address the problem in a cost-effective manner.

Resources

NOSS Statement on Opening ABLE Accounts (December 19, 2016) - http://www.ndss.org/Global/Policy/ABLE/NDSS statement_on opening_ABLE accounts.pdf

Federal ABLE Act - Includes Frequently Asked Questions and "ABLE Questions Answered" video www.ndss.org/Advocacy/legislative-Agenda/Economic-Self-Sufficiency/Achieving-a-Better-life-Experience-ABLE-Act/

ABLE State legislation and Implementation Updates at www.ndss.org/Advocacy/legislative-Agenda/Economic-Self-Sufficiency/Achieving-a-Better-Life-Experience-ABLE-Act/ABLE-State-Bills

State ABLE Programs - Find links to webpages of state ABLE programs at www.ndss.org/Advocacy/Legislative-Agenda/Economic-Self-Sufficiency/Achieving-a-Better-Life-Experience-ABLE-Act/State-ABLE-Programs

ABLE Alliance for Financial Empowerment - Co-founded in February 2016 by NOSS President Sara Hart Weir - www.theablealliance.org

NOSS ABLE 2nd Anniversary Report (December 2016), which includes the status of the federal ABLE Act, the progress of ABLE in the states and the history of ABLE, at http:ljwww.ndss.org/Global/Policy/ABLE/ABLE-Anniversary-Report-online.pdf

ISSUE BRIEF: Health Care Reforms Impacting People with Disabilities

Legislative Request

Please oppose any future changes to Medicaid and the health care system that are detrimental to people with Down syndrome, and support reforms that encourage people with Down syndrome to live and work in their communities, develop assets that reduce dependence on public benefits and avoid costly and segregated institutions.

Background and Summary

Although Congressional leaders on March 24 cancelled a vote in the House of Representatives on H.R. 1628, the American Health Care Reform Act (AHCA), and the legislation is unlikely to be revived in its current form, NOSS is concerned that many of its provision that are detrimental to people with Down syndrome could emerge in future health care reform legislation.

AHCA was intended to repeal and replace the Affordable Care Act (ACA). It did maintain a number of provisions of the ACA that are important to people with Down syndrome, including allowing young adults under the age of 26 to stay on their parents' insurance plans, preventing insurance companies from discriminating against individuals with pre-existing conditions and barring insurance providers from imposing annual and lifetime limits. However, enactment of changes to the structure of the Medicaid program contained in the AHCA would present serious problems for people with Down syndrome and their families. These include:

1. *Per Capita Reimbursements and Block Grants*

The AHCA contains provisions to reimburse states for Medicaid payments based on either per capita-based caps or flexible block grants. Under current law, the financing and administration of Medicaid is shared by the federal government and state governments. Health care providers are generally paid by the states, with a percentage of expenditures being provided by the federal government, regardless of the cost of medical services.

Under the legislation, the federal government would establish, beginning in 2020, a limit on the amount of its reimbursements to the states by establishing a per capita reimbursement cap that would be based on the average per-enrollee cost of medical services in 2016 for each state, or by providing a block grant of funds for a period of 10 years.

The per capita reimbursements would be indexed annually to the increase in the medical care component of the Consumer Price Index (CPI-M), except reimbursements for the elderly and disabled would receive an annual increase of CPI-M plus one percent. The legislation establishes per enrollee reimbursement rates for five specified groups (the elderly, disabled people, children, newly eligible adults, and all other adults). If a state chooses to spend more than the federal reimbursement limit, the state would have to cover the difference.

The amount of block grant funding would be calculated by computing the per capita cost for the eligible population, multiplied by the number of enrollees in the year prior to adopting a block grant. The funding will increase by the growth in the consumer price index but will not adjust for changes in population.

Enactment of Medicaid per capita reimbursement caps and block grants would halt the progress that has been made nationally in promoting home and community-based services, improving the coordination of care and services resulting from medical complexities, and facilitating economic independence for people with Down syndrome and other disabilities. That's because the caps and block grants are intended to slow the rate of federal health care spending, and programs critically important to people with Down syndrome - such as employment supports through Long-Term Services and Supports (LTSS), and Section 1915(c) Home and Community-Based Services (HCBS) - are optional services that states are not mandated to provide.

As a result of such changes to the funding structure of Medicaid, states would likely scale back benefits, impose even longer waiting lists for in-home and community based services and restrict access to needed care for people with disabilities. Equally important, states would have fewer resources to incentivize providers to transform their practices to provide more integrated services and better care coordination, which would help to lower long-term health care costs.

2. Elimination of the Community First Choice (CFC) Option

The ACHA called for the elimination of a provision in the ACA called the Community First Choice (CFC) option, which seeks to reduce state waiting lists for individuals with disabilities to receive at home supports and services by providing states with extra Medicaid federal matching funds to cover the services. This program, which provides opportunities to work in meaningful and competitive employment settings, allows states the flexibility to innovate within their programs and rebalance resources from expensive institutional care into cost-effective community services. Eight states have adopted the CFC option: California, Connecticut, Maryland, Montana, New York, Oregon, Texas and Washington. The states of Arkansas, Colorado, Minnesota, and Wisconsin have applied or are considering the CFC option.

3. Repeal of Essential Health Benefits

The AHCA called for the elimination of a requirement that Medicaid (as well as private health plans) cover 10 Essential Health Benefits (EHBs) proscribed in the ACA, including rehabilitative and habilitative services and devices. These services are particularly important for individuals with Down syndrome, who typically face delays in basic physical, cognitive, language, social and self-help skills. Their having access to early intervention and habilitative services is critical for achieving optimal health outcomes, improving skills and functioning for daily living and becoming active and productive participants in their communities .

Key Messages

Medicaid is a program that reaches far beyond the scope of healthcare for individuals with Down syndrome and encourages people with Down syndrome to live and work in their communities, develop assets that reduce dependence on public benefits and avoid costly and segregated nursing homes or institutions. For example:

- Through Medicaid Long-Term Services and Supports (LTSS), people with Down syndrome can also receive employment supports that enable them to both attain and maintain gainful employment; and

- Home and Community-Based Services (HCBS) provide opportunities for Medicaid beneficiaries to receive services in their own home or community rather than in institutions or isolated settings.

Medicaid is vitally important for people with Down syndrome who generally do not have access to employer-based or other private coverage. Moreover, people with Down syndrome can have significant medical needs, and often require assistance with activities of daily living throughout their lives.

Due to advances in medical technology, individuals with Down syndrome are living longer than ever before. Today, as many as 80 percent of adults with Down syndrome reach the age of 60, and many live even longer. This necessitates access to affordable health care and long-term services and supports throughout an increased lifespan. Unless calibrated to account for both the complicated health care needs and supportive services that people wit h Down syndrome will face throughout their lifespan, the Medicaid reforms in the AHCA will be complex, arbitrary and detrimental to people with Down syndrome.

Answers to Questions about Health Care Reforms Impacting People with Disabilities

1. **The ACHA called for est ablishing a subcategory of reimbursement for people with disabilities, and providing annual increases that would be one-percentage point above medical inflation. Why isn't this per capita reimbursement sufficient to address the needs of people with Down syndrome?**

The indexing of one-percent above medical inflat ion certainly helps, but M edicaid is a program that reaches far beyond the scope of healthcare for individuals with Down syndrome. People with Down syndrome receive employment supports that enable them to both attain and maintain gainful employment. They also receive Home and Community-Based Services (HCBS) that allow them to be active and valued members of their communities. Employment supports and HCBS are *optional services* that cannot be effectively accounted for in the CPI-M, and would likely be the first services to be cut.

2. **The ACHA sought to freeze new enrollments in the ACA's Medicaid expansion. Would this help people with Down syndrome by ensuring that long-term funding is preserved for those who need it most?**

In 2017, the ACA provides a 95 percent match for states that expanded Medicaid for able-bodied adults with incomes un der 138 percent of the federal poverty level. In contrast, states wishing to reduce or eliminate their waiting lists for Section 1915{c) Home and Community-Based Services Waivers receive the normal Medicaid matching rate, which ranges from 50 percent to 75 percent, based on the states' relative income.
Unfort unat ely, the AHCA *did not* maintain the normal matching rate for individuals with disabilities. Therefore, it would do nothing to reduce the Section 191S(c) waiting lists, and would likely cause the waiting lists to increase as reimbursement caps fail to meet the actual costs of providing supports and services to people with disabilit ies.

3. **What other health care reforms would be helpful to people with Down syndrome?**

Enact the Advancing Care for Exceptional (ACE) Kids Act - Because their complex medical conditions can be costly, many children with Down syndrome depend on state-based M edicaid programs. They also require specialized care in centers of excellence, often times outside of their state. Unfortunately, current Medicaid rules can limit access to coordinated care and restrict options to receive medical treatment by out-of-state specialists. The ACE Kids Act is bipartisan legislation that will soon be introduced (see S. 298 and H.R. 546 in the 114th Congress) to create a mechanism for states to participate in a national framework for children with medical complexities to receive cost-effective and coordinated health care and support. This framework could

significantly reduce the necessity for more extensive medical interventions later in life, thus improving the long-term financial viability of the Medicaid program.

Incentivize productivity and work - The current eligibility framework for Medicaid penalizes work and employment for individuals with Down syndrome. Future reforms should incorporate changes that improve opportunities for people with Down syndrome and other disabilities to obtain integrated employment and reduce their relegation to subminimum wages and segregated environments. Medicaid reforms should include incentives for states to meet certain benchmarks for expanding employment opportunities for people with Down syndrome and other disabilities within the state, and offer cost-effective supports and services that promote self-determination, independence, productivity, and integration and inclusion .

Address lifespan needs - Due to advances in medical technology, individuals with Down syndrome are living longer than ever before. Today, as many as 80 percent of adults with Down syndrome reach the age of 60, and many live even longer. This necessitates access to affordable health care and long-term services and supports throughout an increased lifespan. Efforts to reform the Medicaid program should seek to address the gaps and barriers to health care that prevent individuals with Down syndrome from experiencing a high quality of life as they transition from childhood to working adult to senior citizen. This includes access to wellness and prevention services, health and health disparities research, patient-centered care models and increased professional training for health care providers.

Resources

NOSS letter to Members of Congress regarding the ACA and Medicaid, March 8, 2017
www.ndss.org/Global/Policy/ACA/NDSS Congressional_Letter ACA Medicaid-3.10.17.pdf

NOSS Priorities for Medicaid Reform in 2017, February 27, 2017
www.ndss.org/Global/Policy/Medicaid/NDSS_Medicaid_Principles Statement.pdf

"Summary of the American Healt h Care Act," The Henry J. Kaiser Family Foundation, March 6, 2017
files.kff.org/attachment/Proposals-to-Replace-the-Affordable-Care-Act-Summary-of-the-American-Health-Care-Act

Congressional Budget Off ice Cost Estimate of the America n Health Care Act, M arch 13, 2017
www.cbo.gov/sites/default/files/11Sth-congress-2017-2018/costestimate/americanhealthcareact.pdf

List of Medicaid Benefits (Mandatory and Opt ional) at www.medicaid.gov/medicaid/benefits/list-of-benefit s/ in dex.ht ml.

Report of the House Committee on the Budget on the American Health Care Act at
https://www.gpo.gov/fdsys/pkg/CRPT-11Shrpt52/pdf/CRPT-115hrpt52.pdf

ISSUE BRIEF: Congressional Task Force on Down Syndrome

Legislative Request

Please join the *Congressional Task Force on Down Syndrome* by contacting:

- **Senate:** Kyle Christian with Sen. Moran at kyle_christian@moran.senate.gov or (202) 224-6521

- **House:** Megan Perez with Rep. McMorris Rodgers at megan.perez@mail.house.gov or (202} 225-2006

Background and Summary

Established in May 2015, the Congressional Task Force on Down Syndrome is one of nearly 150 informal personal interest Congressional Member Organizations. In general, these organizations focus on increasing public and Congressional awareness of issues, offer new solutions for addressing them and attempt to influence the Congressional agenda.

The Task Force, which is bipartisan and bicameral (includes Members of the House and Senate) is an expansion of the Congressional Down Syndrome Caucus, which was originally formed in 2008 and only included Members of the House.

The Task Force works to increase awareness in Congress about Down syndrome, and to promote bipartisan policies that further the understanding of issues important to individuals with Down syndrome and their families. It does so through various means, including letters to agency leaders, Member and staff briefings on topics of interest, and the dissemination of information about the value and acceptance of people with Down syndrome.

The Task Force has six co-chairs: Senators Jerry Moran (R-KS} and Bob Casey (D-PA); and Representatives Cathy McMorris Rodgers (R-WA}, Cheri Bustos (D-IL}, Pete Sessions (R-TX} and Eleanor Holmes Norton (D-DC).

Key Messages

- Congressional Task Forces and Caucuses provide a forum for discussion and analysis on issues and legislation.

- Members of Congress are not obligated to vote a certain way - joining the Task Force just indicates that they are interested in being supportive of the Down syndrome community and are interested in being kept up-to-date on important issues affecting our community.

- Task Force convenes informational events for Members of Congress and their staff to increase awareness about Down syndrome, and works to advance bipartisan solutions that seek to promote the value, inclusion and independence of individuals with Down syndrome.

- The Task Force regularly partners with NDSS to develop educational, medical and employment related initiatives that support and expand opportunities for individuals with Down syndrome.

- EVERY Representative and Senator should also be a member of the Task Force on Down syndrome - this is an easy, non-controversial Ask!

Answers to Questions about the Congressional Task force on Down Syndrome

1. **If a Senator or Representative was a member of the Task Force in the previous Congress, do they have to sign up again or will their membership automatically roll over to the new Congress?**

House membership in the Congressional Task Force on Down Syndrome does carry over from one Congress to another. However, Senate membership does not, so those who were members in the last Congress need to sign up again .

2. If **the Task Force is informal and does not have any power or authority, why is it important that the Senator or Representative join?**

Down syndrome issues span the lifecycle, from education to economic self-sufficiency, to community integration and employment, to health care and research. There is no other entity in Congress that can take a comprehens ive and coordinated approach to public policies impacting individuals with Down syndrome. In addition, government agencies look to the Task Force as a barometer of support for people with Down syndrome when developing their programs, policies and budgets.

3. **What is the time commitment to serve on the Task Force?**

There is no commitment other than to have a Senator's or Representative's name listed as a member of the Task Force. We would hope the Senator or Representative, and his or her staff, would participate in as many Task Force activities as possible. But we understand that Members of Congress and their staff are stretched thin, and we appreciate any effort that supports the value and acceptance of people with Down syndrome.

Resources

Background on the Congressional Task Force on Down Syndrome and list of members at www.ndss.org/Advocacy/CongressionaI-Task-Force-on-Down-Syndrome

Senate "Dear Colleague" letter at http:ijwww.ndss.org/Global/Policy/CTFDS/CTFDS-Dear Colleague Senate-March2017.pdf

House "Dear Colleague" letter at http:ljwww.ndss.org/Global/Policy/CTFDS/CTFDS-Dear Colleague House-2017.pdf

ISSUE BRIEF: FDA Oversight of Laboratory Developed Tests

Legislative Request

Please support legislative efforts to modernize the regulatory oversight of laboratory developed tests (LDTs) based on the risk-based reforms outlined in the Food and Drug Administration's (FDA's) January 13, 2017 discussion paper, and in the bipartisan draft legislation under development by Reps. Larry Bucshon (R-IN) and Diana DeGette (D-CO).

0 **House:** House offices may express support for the bipartisan Bucshon-DeGette legislative effort by contacting Jeffrey Lucas with Rep. Larry Bucshon at Jeffrey.Lucas@mail.house.gov or (202) 225-4636, and Polly Webster at Polly.Webster@mail.house.gov or (202) 225-4431.

D **Senate:** Senate offices may express support for legislative reform of LDTs in general, or the FDA's January 13, 2017 discussi on paper on risk-based reforms in particular, by contacting the staff of the Health, Education, Labor and Pensions Committee.

Background and Summary

A laboratory developed test (LDT) is a type of in vitro diagnostic test (IVDT) that is designed, manufactured and used within a single laboratory to measure or detect a wide variety of diseases and conditions, including Down syndrome. But these tests are regulated differently depending on whether they are developed and manufactured by FDA-regulated device manufacturers or by certified laboratories regulated by the Centers for Medicare and Medicaid Services under the Clinical Laboratory Improvement Amendments (CUA) Act. As a res u lt , la borato ry developed non-invasive prenat al tests (NIPTs) do not undergo the FDA's ext ensive testing to ensu re their accuracy before being released on the market to impact women and families who consider and/or decide to have a prenatal test for Down syndrome.

The FDA has identified problems with several high-risk LDTs including: claims that are not adequately supported with evidence; lack of appropriate controls yielding erroneous results; and falsification of data. The concern is that people could initiate unnecessary treatment or delay or forego treatment altogether as a result of an incorrect diagnosis. In many instances, for examp le, women are not being told that laboratory developed NIPTs are not regulated by the FDA, and they are usin g the "result s" as a diagnostic rather than a test that requires addit ional screening . Further, these tests are not being cou pled with the necessary and adequate genet ic counsel ing t hat women and famili es require when going through test in g and scr eening for Down syndrome.

In the last Congress, both the House Energy and Commerce Committee and the Senate Health, Education, Labor and Pensions Committee held hear ings on modernizing the regulatory fr amewo rk for LDTs. On January 13, 2017, the FDA issued a discussi on paper that is meant to serve as an approach to Congressional legislation. The paper outlines a risk-based approach to FDA oversight which focuses first on the tests for which the consequences of a false result are known to have the highest risk to the patient. Congressional enactment of legislation to modernize LDT regulat ory oversight will help to promote a consis.tent, predictable and efficient regulatory process that can ensure accuracy and transparency of LDTs.

On March 20, Representatives Larry Bucshon (R-IN) and Diana DeGette (D-CO) released a discussion draft of the Diagnostic Accuracy and Innovation Act (DAIA), which would provide a predictable and timely path to market for innovative diagnostic tests. It would provide a flexible, risk-based approach to the regulation of diagnostic tests that are not medical devices by establishing a separate regulatory structure under the Food, Drug, and Cosmetic Act. The Representatives are requesting feedback and comments on the discussion draft by April 7, 2017.

Key Messages

Expectant parents deserve to receive the most accurate, up-to-date, evidence-based information available when receiving a prenatal diagnosis of Down syndrome. Enactment of reforms outlined in the FDA's discussion paper and in the discussion draft of the Bucshon-DeGette bill would help to accomplish that goal by ensuring that laboratory developed NIPT screenings are safe and effective by among other things, ensuring that claims are adequately supported with evidence, and the tests use appropriate controls to limit erroneous results.

In many instances, patients are not being told that these tests are not regulated by the FDA, and the results are being used as a diagnostic rather than a preliminary screening. The tests that screen for Down syndrome offer an estimation of a baby's chance of having the condition, but families are led to believe that they offer a more accurate diagnostic. Under the regulatory regime established under the Clinical Laboratory Improvement Amendments (CLIA), cell-free DNA prenatal screenings are not all required to meet the FDA's standards of accuracy in testing.

As a result, there is a widespread misunderstanding (often promoted by the laboratories) that genetic indicators identified in the tests can accurately predict Down syndrome and other conditions. But it is increasingly apparent that the limitations and dangers of misinterpretation inherent in these new forms of testing are not stopping their aggressive marketing. As comprehensive genetic screening becomes more standard, this dilemma will only grow and victimize more and more expectant parents into making life-changing decisions based on false positives and inadequate data and information.

The challenges associated with the lack of FDA oversight are compounded by a lack of transparency from providers to patients who are offered cell-free DNA prenatal screenings, typically at ten weeks of pregnancy. In the case of a diagnosis of Down syndrome, there is little or no effort on the part of testing companies and/or providers to present accurate, up-to-date information about the condition prior to, at the time of or after the test results are provided. Rarely are prospective parents told about the positive facts - that, today, people with Down syndrome are going to college, seeking meaningful and competitive employment, living independently, getting married and having all the opportunities to pursue their own hopes and dreams.

Congress needs to carefully consider the consequences of not allowing the FDA to regulate high-risk LDTs, such as cell-free prenatal DNA screenings. As these tests continue to grow and evolve, FDA regulation needs to be there to ensure that the tests being offered are reliable, factual, understood and administered correctly. And when a diagnosis of a genetic condition such as Down syndrome is made, patients need to be provided with accurate and unbiased information about the condition from relevant national disability organizations and medical professional societies.

Answers to Questions about Oversight of Laboratory Developed Tests

1. **What about the concern that FDA regulation is slow and cumbersome, traditionally requiring prospective clinical trial data and a lengthy review process, which limits innovation and could prevent important diagnostics from coming to the market?**

There needs to be a balanced approach that protects public health but does not deter innovation or patient access to testing. The FDA's January 13th discussion paper and the discussion draft of the Bucshon-DeGette bill seek to achieve that balance by outlining a risk-based approach to FDA oversight which focuses first on the tests for which the consequences of a false result are known to have the highest risk to the patient.

2. **How will FDA Oversight of LDTs necessarily improve the way Down syndrome is diagnosed?**

When parents receive a prenatal diagnosis of Down syndrome for their unborn chil d, it is typically delivered with a whole laundry list of medical and behavioral risk factors that accompany the condition. There is usually no mention of the advances in longevity and medical outcomes, and no information about existing support networks that are available to assist families and give them a more balanced, less clinical context in which to consider future prospects. Down syndrome is not a disability to be universally avoided, but that is the way it is treated by physicians. With FDA oversight comes the necessity for proper counseling and more balanced information about the quality of life that people with Down syndrome have today, and the how it will improve in the future.

3. **Is there is enough support in Congress to enact a legislative solution?**

In the last Congress, both the House Energy and Commerce Committee and the Senate Health, Education, Labor and Pensions Committee held hearings on the issues of modernizing the regulatory oversight of LDTs. The FDA's discussion paper, which is based on 300 sets of comments from affected stakeholders, as well as the discussion draft of the Bucshon-DeGette bill, provide a framework for a consensus. We just need Congress to act on it this year so we can address the shortcomings that allow LDTs to be administered with high rates of false positi ves and false negatives , and without providing expectant parents with the most accurate, up-to-date, evidence-based information available when receiving a prenatal diagnosis of Down syndrome.

Resources

NDSS Position on Down Syndrome Prenatal Testing at www.ndss.org/About-NDSS/Media-Kit/Position-Papers/National-Down-Syndrome-Society-Organizational-Position

FDA Discussion Paper on Laboratory Developed Tests at www.fda.gov/downloads/MedicalDevices/ProductsandMedicalProcedures/lnVitroDiagnostics/LaboratoryDevelopedTests/UCM536965.pdf

NDSS comments on the Proposed LDT Regulatory Oversight Framework at www.ndss.org/Global/Policy/NDSS_statement_on proposed_FDA oversight_of LDTs.pdf

Bucshon-DeGette Press Release on Diagnostic Accuracy and Innovation Act at bucshon.house.gov/media-cent er/ press-releases/ dr -bucshon-degett e-release-draft-diagnostic-accuracy-and-innovation-act

ISSUE BRIEF: Congenital Heart Defect Research

Legislative Request

Please cosponsor H.R. 1222/S. 477, the Congenital Heart Futures Reauthorization Act.

D **Senate:** Senate offices may cosponsor by contacting Jessica McNiece with Senator Richard Durbin at jessica_mcniece@durbin.senate.gov or (202) 224-2152.

D **House:** House offices may cosponsor by contacting Shayne Woods with Rep. Gus Bilirakis at Shayne.Woods@mail.house.gov or (202) 225-5755.

Background and Summary

On February 27, Representatives Gus Bilirakis (R-FL) and Adam Schiff (D-CA) introduced H.R. 1222, the Congenital Heart Futures Reauthorization Act. A companion bill, S. 477, was introduced in the Senate on March 6 by Senators Richard Durbin (D-IL) and Bob Casey (D-PA). The bills seek to expand research for congenital heart defect (CHD) treatments and encourage lifelong, specialized care for patients.

CHDs are problems with the heart's structure that are present at birth. They are the most common birth defect, impacting about 40,000 babies each year, and it is estimated that 2-3 million adults and children are living with CHDs in the US. Approximately one-half of all children with Down syndrome are born with CHD, most commonly Atrioventricular Septa! Defect, Ventricular Septal Defect, Persistent Ductus Arteriosus and Tetralogy of Fallot. Although common defects can be repaired with a limited risk of death, CHD in people with Down syndrome during the early years of life has the potential to significantly affect cognitive function and overall health status later in life, and necessitates extensive medical intervention. In fact, neurodevelopmental outcomes among children with Down syndrome are known to be worse than those of typically developing children who have the same heart defects.

First enacted into law in 2010, the Congenital Heart Futures Act has done much to improve the nation's surveillance, research and education efforts to fight CHD. The law expanded infrastructure to track the epidemiology of CHD at the Centers for Disease Control (CDC) and increased lifelong CHD research at the National Institutes of Health (NIH). Since enactment of the Congenital Heart Futures Act, Congress has appropriated $11 million to the CDC for these activities. The original law also urged the National Heart, Lung and Blood Institute (NHLBI) to continue its use of its multi-centered congenital heart research network, the Pediatric Heart Network (PHN), to help guide the care of children and adults with CHD. Together, these efforts have improved our understanding of CHD across the lifespan, the age-specific prevalence and factors associated with dropping out of appropriate specialty care.

The Congenital Heart Futures Reauthorization Act will further enhance the CDC's surveillance of CHD, and promote NIH research, public outreach and education. It would do so by:

- **Assessing the current research needs and projects related to CHO across the lifespan at the NIH** -The bill directs the NIH to assess its current research into CHD so that we can have a better understanding of the state of biomedical research as it relates to CHD.

- **Expanding research into CHO** - The bill directs the CDC to continue to build their public health research and surveillance programs. This will help us understand healthcare utilization and

demographics, and lead to evidence-based practices and guidelines for CHD.

- **Raising awareness of CHO through the lifespan** -The bill allows the CDC to establish and implement a campaign to raise awareness of congenital heart disease. Those who have a CHD and their families need to understand their healthcare needs, and promote the need for pediatric, adolescent and adult individuals with CHD to seek and maintain lifelong, specialized care.

Key Messages

Although common congenital heart defects can be repaired with a limited risk of death, research shows that CHD in people with Down syndrome during the early years of life has the potential to significantly affect cognitive function and overall health status later in life, and necessitates extensive medical intervention. In fact, according to the NIH Health Research Plan on Down Syndrome, neurodevelopmental outcomes among children with Down syndrome are known to be worse than those of typically developing children who have the same heart defects.

Down syndrome is a major cause of congenital heart disease, affecting about 5,000 of the 40,000 babies born each year with CHD. The overall incidence of congenital heart disease is 0.8 percent in the general population, but about 50 percent in children with Down syndrome. It is imperative that people with Down syndrome be fully included in any research focused on diagnosis, treatment, prevention and long-term outcomes of CHD.

Because CHD is so prevalent in people with Down syndrome, increased federal research focused on diagnosis, treatment, prevention and long-term outcomes of CHD in the Down syndrome population could potentially provide important insights into the cause or causes of the disease and intervention strategies for the broader population.

Answers to Questions about the Congenital Heart Futures Reauthorization Act

1. What is the relationship between Congenital Heart Disease and Down syndrome?

Congenital heart defects can be related to a number of factors, including chromosome abnormalities, gene defects or environmental factors. Problems with chromosomes that result in genetic syndromes, such as Down syndrome, often result in a higher incidence of infant heart malformations. Chromosome abnormalities associated with congenital heart defects, in addition to Down syndrome, include Trisomy 18, and Trisomy 13, Williams syndrome, Turner's syndrome, Cri-du-chat syndrome, Wolf-Hirschhorn syndrome and DiGeorge syndrome.

2. How will the Congenital Health Futures Reauthorization help people with Down syndrome?

Life expectancy for people with Down syndrome has more than doubled in the past 30 years as a result of medical advances in screening for and repairing congenital heart defects. The issue today is the quality of that extended life. Congenital heart disease during the early years of life has the potential to significantly affect cognitive function and overall health status later in life, thus necessitating extensive medical intervention. The extended research and surveillance programs proposed in the legislation will provide better information about the most effective medical interventions.

Resources

The Heart and Down Syndrome, NOSS at http://www.ndss.org/Resources/Health-Care/Associated-Conditions/The-Heart--Down-Syndrome/

NIH Health Research Plan on Down Syndrome, page 8, at www.nichd.nih.gov/publications/pubs/Documents/DSResearchPlan_2014.pdf

Pediatric Congenital Heart Association at http://conqueringchd.org/

Adult Congenital Heart Association at https://www.achaheart.org/

ISSUE BRIEF: NIH Research on Down Syndrome

Legislative Request

Urge the National Institutes of Health to allocate additional resources toward expediting the research priorities identified in the NIH Research Plan on Down Syndrome.

Background and Summary

The NIH Research Plan on Down Syndrome is an 80-page scientific plan that outlines Down syndrome research goals and objectives for the NIH Institutes. The plan was developed by the NIH Down Syndrome Working Group, which includes representatives from 11 NIH Institutes, Centers and Offices. The Group consulted with the scientific, family and advocacy communities in drafting the plan, which was updated in December 2014.

The Research Plan on Down Syndrome has been an important catalyst for bringing the Down syndrome research community together around focused priorities. It provides a clear roadmap for how studying many of the coexisting conditions in Down syndrome, such congenital heart disease and mental health disorders, as well as studying conditions that are rare in people with Down syndrome, such as solid tumors. This research can provide new insights into how best to treat all people with those conditions, not just those with Down syndrome.

In recent years, NIH has supported a number of initiatives that expand our understanding of Down syndrome, not only in the area of Alzheimer's disease, but other associated medical and psychiatric conditions. This includes funding the "Biomarkers of Alzheimer's Disease in Down Syndrome" initiative; working with the Down syndrome research community on the development of outcome measures for cognitive, behavioral, and physical measures for clinical trials; and coordinating with the NIH Down Syndrome Working Group to launch OS-Connect®, the Down syndrome patient registry.

NIH received a $2 billion increase in funding for FY 2016. In addition, the 21st Century Cures Act enacted by Congress as the end of 2016 provided more than $4 billion more in additional funding for the NIH spread over five years. Although NIH funding for fiscal year 2017 has remained frozen for the first five months, Congressional appropriators are reportedly considering an increase in the NIH budget for the remainder of the fiscal year, as well as for fiscal year 2018.

Key Messages

---)- NIH should be commended for developing the Research Plan on Down Syndrome and supporting recent initiatives such as the Alzheimer's biomarkers study and the patient registry (OS-Connect®).

---)- However, much more needs to be done to fulfill the objectives outlined in the Research Plan. This includes expanding cross-institute collaboration to address the persistent challenges that impede the development of clinical and behavioral treatments across the lifespan of people with Down synd rome.

---)- Currently, Down syndrome research is primarily the purview of the National Institute of Child Health and Human Development . However, Down syndrome is no longer a childhood condition. Some individuals with Down syndrome are living to be nearly 80 years old, and there is much to learn from and about the medical and behavioral conditions that are associated with Down syndrome.

Answers to Questions about NIH Research on Down Syndrome

1. How is increased NIH funding for Down syndrome research a good investment?

In addition to improving the quality of life for people with Down syndrome, studying many of the coexisting conditions in Down syndrome, such as Alzheimer's disease, autism spectrum disorders and congenital heart disease, will provide new insights into how best to treat all people with those conditions, not just those with Down syndrome. The same holds true for studying conditions that are rare in people with Down syndrome, such as solid tumors. Take Alzheimer's, for example. The majority of individuals with Down syndrome develop the pathology of Alzheimer's disease while in the mid-40s. Understanding the disease process in people with Down syndrome could help in the discovery of new drugs and other treatments for others with or on the path to Alzheimer's disease.

2. How will increased NIH funding for Down syndrome research improve the quality of life for people with Down syndrome?

People with Down syndrome experience cognitive delays, but the effect is usually mild to moderate and is not indicative of the many strengths and talents that each individual possesses. Quality educational programs, a stimulating home environment, good health care and positive support from family, friends and the community enable people with Down syndrome to lead fulfilling and productive lives.

At the same time, people with Down syndrome have an increased risk for certain medical conditions such as congenital heart defects, respiratory and hearing problems, Alzheimer's disease, childhood leukemia and thyroid conditions. Many of them also have secondary neurobiological, behavioral and psychological conditions, such as Autism spectrum disorders. With increased NIH funding for Down syndrome research, many more of these conditions can be treatable so that people with Down syndrome can live more fulfilling lives. This includes attending college and going to work, participating in decisions that affect them, having meaningful relationships, voting and contributing to society in many wonderful ways.

3. If more funding were provided to Down syndrome research by the NIH, what priorities should come next?

First, Down syndrome researchers and health care providers would benefit significant ly from a longit udinal study of people with Down syndrome across the lifespan to gather natural history data and to determine the effects of pharmaceutical and behavioral interventions.

Second, researchers could use NIH help in setting up one or more brain and tissue biorepositories for Down syndrome to systematically collect, store and distribute brain and tissue samples to Down syndrome researchers.

Third, more resources are needed to address the behavior health needs of people with Down syndrome, including those with Autism spectrum disorders, with the goal of providing support to those afflicted with such conditions.

Resources

NDSS Brief on Down Syndrome Research and Funding at http://www.ndss.org/Advocacy/Legislative-Agenda/Health-Care-Research/Biomedical-Innovation-Research/Down-Syndrome-Research-and-Funding

Down Syndrome Directions: The National Institutes of Health Research Plan on Down Syndrome at https://www.nichd.nih.gov/news/resources/spotlight/Pages/120814-DS-research-plan.aspx

NIH Down Syndrome Consortium and Working Group at https://downsyndrome.nih.gov/Pages/default.aspx

NIH OS/ Alzhei mer's Biomarkers Study Grant at https://grants.nih.gov/grants/guide/rfa-files/RFA-AG-15-011.html

Press Release: National Institutes of Health Establishes Down Syndrome Patient Registry at https:ljwww.nih.gov/news-events/news-releases/nih-establishes-down-syndrome-patient-registry

National Institutes of Health Forms Down Syndrome Consortium with NDSS and Other Down Syndrome Groups at https://www.nih.gov/news-events/news-releases/down-syndrome-consortium-formed