

State of Connecticut
GENERAL ASSEMBLY



PUBLIC HEALTH COMMITTEE
LEGISLATIVE OFFICE BUILDING, ROOM 3000
HARTFORD, CT 06106-1591
TELEPHONE (860) 240-0560 FAX (860) 240-5306

TASK FORCE TO STUDY RARE DISEASES

Meeting Summary

June 23, 2017

Meeting called to order by Dr. Mridu Gulati at 1:10pm.

Members Present: Dr. Mridu Gulati, Dr. Cathy Lutz, Dr. Joseph Tucker, Lynne Sherman, Nancy Dupont, John Morthanos, Michelle Cotton and Lesley Bennett.

Members Absent: Dr. Barbara Pober, Dr. Brian Smith, Dr. Michele Spencer-Manzon and Paul Pescatello

Others Present: Dr. Robert Zavoiski and Kathy Bruni, Department of Social Services; Maryann May, Connecticut Hemophilia Society, Inc.; and Virginia Pertillar, Citizen for Quality Sickle Cell Care.

Dr. Gulati welcomed everyone and announced that Kathy Bruni from the Department of Social Services (DSS) is in attendance to present on the Medicaid Waiver Program. She added that she would like to revisit previous meetings and talk about some of the topics, themes and issues that were discussed and to take some time to potentially establish some subcommittees and tangible goals.

Dr. Gulati stated that a lot of time is spent talking about Newborn Screening and she believed it's a good place to start since there is a lot of data housed within the State. She added that one theme that came up is services which are relevant for other rare disease pediatric populations. She pointed out that the group did not talk much about the adult population and that any unique needs for the adult population that are different should be addressed.

Dr. Gulati listed the following as topics/themes from previous meetings: community outreach and outreach to the providers both in terms of medical education and awareness of resources, streamlining things and improving infrastructure. She stated that it is important to think about who would make up the subcommittees, and which specialty institution to invite to talk about the difficulties or challenges they experience.

Dr. Gulati stated that the individual and caregiver stories will help to establish the subcommittees and set goals for the task force in terms of recommendations for systems improvement.

Lesley Bennett stated that “transition to adult care” or “transition to adult services” is another issue. She added that institution such as Yale and CCMC are running into problems with transitioning children into adult care. This she said is a nationwide problem and it is the hope of the rare disease community that the task force will address it.

Dr. Gulati asked the group to think about future meeting dates and future speakers. She informed the group that Dr. Pober is still interested in participating but is not able to attend today’s meeting.

Beverley Henry informed the meeting that the new legislation amended the 2015 Public Act and included language into HB 7222 (PA 17-146). The new reporting date is January 1, 2018 and the chairpersons of the Public Health Committee or their designees were added to the membership.

Presentation By: Kathy Bruni, Program Manager, Department of Social Services (DSS)



AN OVERVIEW OF
1915C MEDICAID WA

Please see the link above for the presentation. The following areas were discussed:

- Purpose of a Waiver
- What is a 1915C Waiver
- Waiver requirements
- Waiver assurances to CMS
- Potential target groups
- Relationship with other state agencies
- Waiver process
- Waiver eligibility
- Katie Beckett Waiver
- PCA Waiver
- ABI Waiver
- DDS Waivers & DDS Services
- Autism Waiver & Early Childhood Autism Waiver
- Mental Health Waiver
- CT Home Care Programs for Elders
- Functional and Financial Eligibility
- 1915i State Plan Option
- Program Services
- CMS Final Rules

Discussion

Lesley Bennett reminded the group that Dr. Zavoski had stated that if a medication was available for certain conditions the DSS/Medicaid would pick up the costs. She added that families with insurance are running into problems because some drugs are classified as

specialty care, which is causing families to become insolvent trying to cover the costs. She asked if there is a waiver that the state could qualify for in order to help these families. She added that there is a Connecticut law that requires coverage for medical food but that at age 13 it is no longer covered by insurance.

Dr. Zavoski stated that Katie Beckett is the option for kids with special needs such as medical food and are not Medicaid eligible. He added that there is a 3year waitlist and recommended that anyone needing services should get on the list as soon as possible.

Leslie Bennett asked if the waiting list is the only way.

Dr. Zavoski responded that unfortunately there is a limited budget and the needs are more than there are resources but DSS put as many people on as they can. He stated that 60% of the people with commercial insurance don't really have insurance. He added that it should be on the record that those insured by their employer should have a conversation with their employer to find out why somethings are covered or why others are not.

Lesley Bennett stated that a dietitian at a university hospital informed her that they had a family where the medical food for their child was no longer covered. She added that whenever these children don't get their required medical food they end up in the ICU at a cost up to \$50,000.

Dr. Zavoski agreed and pointed out that current discussion on the healthcare legislation is forcing DSS/Medicaid to carefully evaluate how to do what needs to be done. He added that there will be a cost cap and an increase.

Lesley Bennett inquired if DSS was running into problems with the "specialty tier".

Dr. Zavoski informed her that DSS does not have a specialty tier, but that there are medications costing over half a million dollars per year and the prices keep going up. He stated that some medications don't have much of an impact and that there will be moral questions to wrestle with if a "cap" goes into effect.

Lesley Bennett stated that more diseases are in the pipeline to be added to the Newborn Screening Program in the next few years that will allow for quick diagnoses and treatment. She added that almost all will cost at least \$100,000 per year and families can't afford it.

Dr. Zavoski acknowledged that some are treatments, but for some the treatment impact is small to negligible. He added that a lot of people will be scratching their heads trying to figure this out.

Lesley Bennett stated that struggling parents are debating whether one of the two working household members should give up their job in order to qualify for Medicaid. She added that it's an issue that will put the responsibility on the state.

Dr. Gulati asked if there is an income requirement for all the waivers and if there is any waiver other than Katie Beckett to help people that are going broke

Kathy Bruni responded that if the question is about children, it would be Katie Beckett. She added that for adults the income is \$2205 per month and asset \$1600 and that they could qualify for any of the waivers depending on needs and requirements.

Dr. Gulati inquired if there are empty slots available on any Waiver.

Kathy Bruni responded no, and advised that the Elder Waiver is open for intake.

Dr. Gulati asked about the process for participants to get on a Waiver.

Kathy Bruni stated that provider makes the referral to the DSS and added that the information is on the DSS website. She clarified that the person has to be in a home or community setting in order to qualify for the waiver and added that if the child is in the hospital or is being discharged from the hospital, they will not qualify.

Lynne Sherman inquired if the Waiver is fully funded by the Federal Government or is it split with the State. She asked how the Agency prioritize and shift funding around, when appropriate, with the increase demand and the improved ability to diagnose and treat children at early ages. She pointed out that the statement “that drugs don’t improve outcomes and children’s ability to have quality of life” is a blanket statement because we are seeing unbelievable improvement in their mobility, care and quality of life. She provided an example of a case in which a commercial insurer had approved a drug for five treatments and the patient was doing well until the insurer cancelled coverage in the middle of the treatment. She stated that drug approval process and the impact it has is significant.

Dr. Zavoski stated that while many of these medications do have an impact a lot of them don’t. He added that the DSS is required to pay for any drugs approved by the FDA and that having to decide who gets the drugs and who doesn’t is a decision DSS don’t want to make. He stated that this is a social discussion and our society is not looking at that.

Kathy Bruni added that it’s a challenge because resources are limited and it has to be equitably distributed through a process with the hierarchy of the agency, the Office of Policy and Management and in consultation with the legislative committees of cognizance. She stated that over the years the Katie Beckett Waiver increased from 200 to 300.

Dr. Zavoski added that DSS recently developed and implemented programs to help providers recognize that more kids transitioning to adulthood are having trouble finding providers. He highlighted the PCMH Plus Program which is to incentivize larger providers and FQHCs to work on transition planning or to create adult care system to keep patients in their practice.

Dr. Gulati asked what the Task Force can do to help the above efforts.

Kathy Bruni suggested that members discuss their priorities with their legislators and added that there are advocates in state government for people in need of services.

Dr. Zavoski added that members should contact their legislators in Washington. He also pointed to the cost trends over the last 50 years and added that Medicaid is being relatively flat. He added that Medicaid has been a very good fiscal steward while caring for 1 in 5 or 1 in 4 children. Dr. Zavoski expressed concerns that tough times will be created by cutting people off Medicaid.

Dr. Gulati stated that the responses to the meeting availability survey indicated that Friday afternoons were best for most members. She added that the conference call number will remain the same and that there is an effort to secure the ability to use Video conferencing. She reminded member to look at the themes from previous meetings as a way for planning the next set of meetings and invited speakers.

Dr. Gulati asked if the group should come up with a 4 month plan that would include potential speakers, subcommittees or working groups, additional material to work with and things that are somewhat low-hanging fruit that can actually make a very quick positive difference.

Lesley Bennett informed the group that the DPH is updating their Newborn Screening website and that they are reaching out to other states to assess their system. She stated that legislation will be needed in order to add new tests to the panel and that DPH will need the help of the task force members to approach the Legislature. She added that there are approximately 10 new tests in the pipeline which would allow kids to be diagnosed quickly.

Lesley Bennett suggested that getting Yale, UConn, Connecticut Children Medical Center and some of the larger hospitals involved would be a good way to start educating their people.

Michelle Cotton stated the DPH should add conditions that are life threatening to the test panel. She added that she was unable to find information when her son was diagnosed and that it would be a good idea to indicate if the condition is life threatening. She stated that education and the ability to identify conditions are her two main reasons for serving on the Task Force.

Lesley Bennett announced that there is ongoing discussion to develop an information network. She pointed out that Connecticut is between New York and Boston and that it would be beneficial to establish relationships with both since they are two great areas for dealing with a lot of these issues.

Dr. Joe Tucker stated that there are opportunities to make improvements in the flow of information, especially for families hearing that their child screened positive for a disease.

Dr. Gulati suggested that it might be a good idea to invite the DPH to come back to the task force and tell the group what they have done on Newborn Screening.

Lesley Bennett informed the group that she will make that request when she attends the DPH meeting in July. She stated that the DPH is anxious to work with the taskforce after they realized that we understood the issues, and they were unaware of the lack of pediatrician for many of the diseases. She added that it was surprising to DPH that when people reach-out to the agency there is no one there to help them.

Lynne Sherman commented that if the Task Force is trying to make a determination, there cannot be an assumption that there are some breakdown and issues before reaching out for some feedback from the group we would assess. She added that there are leaders of pediatricians with access to that information and that is a very important piece. She pointed out that some areas of focus should be: when do you connect patients to specialty care, what are the challenges and what are the reasons.

Lynne Sherman further stated that if the focus is on areas where there are opportunities, the questions are: do we really know what providers are doing, do we know what is working well and what opportunities are there to make changes and improvements. She added that connecting the first two is important and that having a better understanding of these areas will help the group make recommendations that will help to validate our position.

Dr. Gulati offered to share the Lupus Survey with everyone, and added that surveys are generally very simple and it can be sent out electronically to the selected groups.

Lynne Sherman pointed out that often times stuff such as surveys are coming at providers and sometimes they see it and just pass it by. She added that having a champion who has regular educational forums with providers present the content of the survey would be a great opportunity with a captive audience.

Dr. Gulati agreed that relationship is important and that she was looking at the community relation for pediatrics for an example of a personalized letter. She admitted that nobody opens letters anymore but if they get a personalized email survey it might help.

Maryann May informed the group that the Connecticut Hemophilia Society recently sent out a short survey to their community and how difficult it was to get people to respond.

Dr. Gulati stated that she didn't believe there is an expectation for a very high response rate to surveys and that the report will reflect the response rate. She added that another area of focus is developing a survey for community providers, and she asked if the group wanted to divide the community providers from individuals at the specialty centers or experts in terms of gathering information.

Dr. Gulati then asked for topics for the next few meetings and suggestions for future speakers. She reminded the group that schools and commercial insurance were already suggested and she asked members to email a general list of questions to be forwarded to the presenters prior to the meetings.

Lynne Sherman stated that there is a lot of work being done, but that it is another bucket when you start to talk about transition like medical home models and pediatrician to adult provider which is interrelated in the triangle with the specialist.

Dr. Gulati stated that because transition interconnects so many people perhaps it is a good idea to have a conversation amongst all the interested parties and added that the Connecticut State Medical Society should be present for the conversation dealing with Medical Homes.

Lynne Sherman stated that there are medical homes in our regions and asked if any are advanced and are performing well that would be willing to present to the group.

Dr. Gulati responded that this is something to investigate.

Lesley Bennett explained her difficulty of finding an adult primary care physician. She informed the group of a recent trend where some pediatric specialist across the country are becoming birth to death providers. She added that Washington National is now in the process of building a special hospital for birth-to-death in order to facilitate children with genetic disorders that can't transition. She also mentioned how the pediatric group that cares for her daughter had offered to continue care but then they decided to go Concierge and is not Medicaid Certified. She stated that this is a big problem and for some parents it's going to get worse as children with rare diseases start aging out.

Dr. Gulati listed schools, commercial insurance and medical homes as suggested areas for presentations. She then asked how the Lupus Survey was distributed and was informed that it was done by UConn. She added that although all the questions on lupus are not relevant, it provides an idea of what the survey would potentially look like.

Lesley Bennett asked if it isn't going to be a little difficult for rare diseases because there are educational materials. She pointed out that ALD is going to vary greatly from Cystic Fibrosis.

Dr. Gulati listed the following as sample questions: do you have timely access to a specialty center, and would you like more information on more rare diseases. She added that the questions will have to be very generic on rare diseases and that maybe it should be divided by pediatric and adults.

Lesley Bennett stated that one of the key questions should be how many of them have a child with rare disease.

Dr. Gulati agreed and added that another question should be, who do they refer the children to.

Lesley Bennett stated that other questions to ask are, what type of rare disease and where do they find information.

Dr. Gulati suggested asking, what types of information would be most helpful and, are they aware of the centers. She stated that pediatric have been the main focus, and added that the group will also have to focus on the adult population.

John Morthanos referenced the earlier comments on "birth-to-death facilities" and stated that there is a much older society. He added that Oregon is the only state that legalize euthanasia and asked if there could be a conversation or phone call to help members evaluate the issue in order to make a recommendation to the state if it's an alternative for very sick people who may want to consider it.

Lesley Bennett agreed that the topic should be address by the Task Force and added that it is difficult for a lot of families to talk about it.

Dr. Gulati reminded the group that there is palliative service.

Lesley Bennett agreed but added that palliative care is not just hospice and that rare diseases communities should be educated on the topic because it works for a lot of the people in the rare disease community.

Michelle Cotton reminded the group that a decision was made in a previous meeting to invite the School Nurses Association to make a presentation to the Task Force. She added that some school nurses are very good dealing with children with rare diseases but others are not. She pointed out that in many cases the 504 Plans are not being followed. She gave an example of an instance where a child had received a prescription for an Air Conditioner and because it was not filled the child suffered a set-back and ended up in the hospital.

Beverley Henry reminded the group that they had talked about the possibility of inviting the Office of Early Childhood and the State Department of Education. She further recommended that the Task Force focus on early childhood and missed diagnoses together and then move on to discuss adulthood and other subject. She also asked the group to put together a list of organizations that they would like to present at task force meetings and arrange them in groups to allow for consistent conversations,

Lesley Bennett informed the meeting that the State Department of Education had developed a packet for rare disease parents, and that her daughter was one of the first to have an

individualized healthcare plan. She suggested that the Task Force have a conversation with the State Department of Education about developing a standardized packet for rare diseases, which would be similar to one for food allergies.

Virginia Pertillar commented that after listening in on the meeting she recognized that with all the efforts to bring awareness to the rare disease communities there is a lack of communication and collaboration between all the rare disease groups. She added that sickle cell is known as a medallion rare disease with over 100,000 diagnosed cases, with approximately 1600 in the State of Connecticut and another 11,000 individuals who carry the traits. She stated that with communication and education the prevalence of the disease will continue showing up in Newborn Screenings.

Virginia Pertillar addressed the issue of the 504 Plan and pointed out that most of the people attending the meetings do not have the full knowledge about sickle cell disease and many other disorders. She pointed to the high incident of substitute teachers entering the classrooms that are not made aware of children with accommodations, and that this shows the breakdown in communication for the staff at a particular school and those who comes in to substitute.

Virginia Pertillar stated that educating the legislature is a must and that it should be done Collaboratively, because no one modality of incident or rarity of disease is more important than the other.

Dr. Gulati announced that she will send the next meeting dates to members. She thanked everyone and adjourned the meeting.