

The Voice of The Patient

A series of reports from the U.S. Food and Drug Administration's
Patient-Focused Drug Development Initiative

Autism

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Center for Drug Evaluation and Research (CDER)
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Introduction

On May 4, 2017, FDA held a public meeting to hear perspectives from individuals with autism, caregivers and other patient representatives on the most significant health effects and currently available therapies for autism. FDA conducted the meeting as part of the Agency's Patient-Focused Drug Development initiative, an FDA commitment under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V) to more systematically gather patients' perspectives on their condition and available therapies to treat their condition. As part of this commitment, FDA is holding 24 public meetings between Fiscal Years 2013–2017, each focused on a specific disease area. The public meeting on Patient-Focused Drug Development for Autism was the 22nd public meeting of this commitment.

More information on this initiative can be found at

<https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm529043.htm>

Overview of Autism

Autism or autism spectrum disorder (ASD) is a complex neurodevelopmental disorder that affects an estimated 1.5% of children in across the United States.¹ Autism is 4.5 times more common among boys (1 in 42) than among girls (1 in 189).¹ Signs of autism can sometimes be detected as early as 18 months; however, many children are not diagnosed until they are much older. As there is no medical test for diagnosis, autism is diagnosed based on behavior and developmental screening.

Autism may manifest as communication, behavioral, and social challenges that range from mild to severely debilitating in nature. As autism is a wide-spectrum disorder, two individuals with autism may present with very different behaviors, development levels, and health or support needs. Behaviors observed in children and adults with autism may include: trouble relating to others or lack of interest in people, extreme sensitivity to certain sounds, smells or lights, repetitive behavior pattern, having narrow obsessive interests, avoiding eye contact, difficulty adapting to changes in routine, and difficulty expressing their needs. Individuals with autism may also experience co-occurring conditions such as depression, anxiety, insomnia or mood disorders.

There is no cure for autism. Currently, drugs are approved for treatment of irritability associated with autism; however, no drug therapies currently target the underlying causes or core manifestations of autism. FDA-approved products for irritability associated with autism include risperidone and aripiprazole. The mainstays of treatment for autism are behavioral therapy and educational interventions. For children, behavioral therapies in the form of early intervention programs often emphasize play-based interactions that encourage shared attention and communication. For adults, services are more vocational and community-based, such as social skills workshops and support groups.

Meeting overview

This meeting provided FDA the opportunity to hear directly from individuals, caregivers, and other stakeholders about their experiences and perspectives with autism. The discussion focused on two key topics: (1) health effects and daily impacts of autism that matter most to individuals and families and (2)

¹ Center for Disease Control and Prevention. Autism Spectrum Disorder: Data & Statistics. Accessed December 14, 2017. <https://www.cdc.gov/ncbddd/autism/data.html>

individuals' and families' perspectives on current approaches to managing autism. FDA was particularly interested in hearing aspects of autism that posed the greatest impact on quality of life. The questions for discussion (Appendix 1) were published in the [Federal Register](#) notice that announced the meeting.

For each topic, a panel of individuals with autism and caregivers (Appendix 2) shared comments to begin the dialogue. Panel comments were followed by large-group facilitated discussions inviting comments from other individuals with autism, affected family members, and caregivers in the audience. An FDA facilitator led the discussion, and a panel of FDA staff (Appendix 2) asked follow-up questions. Participants who joined the meeting via the live webcast (referred to in this report as web participants) were also able to contribute comments. In addition, in-person and web participants were periodically invited to respond to polling questions (Appendix 3), which provided a sense of the demographic makeup of participants and how many participants shared a perspective on a given topic.

Approximately 32 individuals with autism or family members attended the meeting in person. Approximately 161 people attended the meeting through the live webcast, of which 28 people who identified as either individuals with autism or family members contributed input. Per their responses to the polling questions, web participants represented a range of individuals with autism or family members. Most web participants identified as a family member of an individual with autism, and identified themselves or their loved one with autism as male. Web participants ranged in age from younger than 5 to 39 years old. Although participants in this meeting may not fully represent the diverse population of individuals with autism, their input reflected a range of experiences with autism impacts and treatments. The FDA facilitator noted during the meeting the emergence of a variation in perspectives among teens and adults with autism; parents of pediatric children with autism; and parents of adult children with autism, many of whom are unable to live independently. Throughout the meeting, the facilitator attempted to solicit these three perspectives on various topics.

To supplement the input gathered at the meeting, individuals with autism, family members, and others were encouraged to submit comments on the topic to a public docket,² which was open until July 5, 2017. Forty-one comments were submitted to the public docket, the majority by individuals with autism and family members.

More information, including the archived webcast and meeting transcript, is available on the meeting website: <https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm529043.htm>

Report overview and key themes

This report summarizes the input shared by individuals with autism, families, and other autism stakeholders during the meeting or through the webcast. It also includes a summary of comments submitted to the public docket. To the extent possible, the terms used in this report to describe specific health effects, impacts, and treatment experiences reflect the words used by in-person participants, web participants, or docket commenters. The report is not meant to be representative in any way of the views and experiences of any specific group of individuals or entities. There may be health effects, impacts, treatments, or other aspects of autism that are not included in this report.

² A *docket* is a repository through which the public can submit electronic and written comments on specific topics to U.S. federal agencies such as FDA. More information can be found at www.regulations.gov.

The input from the meeting and docket comments underscore the diversity in experiences with autism, the complexity of selecting an appropriate treatment and the broader challenges people with autism face in accessing needed care and support. Several key themes emerged from this meeting:

- Experiences and perspectives on the health effects of autism vary widely. Health effects indicated by meeting participants included cognitive impairments, social difficulties, behavioral issues, insomnia, gastrointestinal symptoms, and inability to adjust to changes in daily routines. Individuals with autism and family members particularly highlighted the impact of disruptive, self-injurious, and aggressive behaviors such as kicking or pushing. Participants noted these behaviors exceed what is often characterized as “irritability” in autistic individuals. Participants also shared debilitating impacts of aggressive behavior on interpersonal relationships, social interactions, and ability to live independently. Participants indicated a broad range of causes for behavioral issues such as communication difficulties, medication side effects and co-occurring conditions, such as anxiety and depression.
- Many types of repetitive behaviors are not bothersome to individuals with autism. *Stimming*, a term that describes behaviors such as hand flapping, tapping, or pacing, was characterized as calming and helpful. Stimming was highlighted as calming during moments of sensory overload or anxiety. A few caregivers highlighted the stigma associated with *stimming* behaviors which may lead to bullying or socially awkward behavior.
- Individuals with autism also manage co-occurring conditions such as depression, anxiety, and mood disorders. Although meeting participants acknowledged that effects of autism and co-occurring conditions are often intertwined, many denoted co-occurring conditions as negatively impacting their health and quality of life more so than autism. As one participant shared, “I don’t feel my autism gets better or worse. But my anxiety does get better or worse.” Meeting participants noted improvements in communication and ability to perform daily activities when co-occurring conditions are well-managed. Participants also highlighted the need for treatments that better address co-occurring conditions.
- Autism affects all aspects of daily life for individuals with autism and caregivers. The complex treatment needs and health effects of autism can limit the ability to perform daily activities, pursue careers and interact socially. Caregivers highlighted the toll of autism on family dynamics due to strict treatment regimens, troublesome behavior, and constant attention needed to care for an individual with autism. Meeting participants also described how autism can significantly impact the ability to live independently due to broader issues such as employment and availability of support services.
- Treatment decisions in autism are highly individual and personal. Participants often described a trial and error process of selecting appropriate treatments. Participants expressed concerns regarding medication side effects. This concern was particularly highlighted in caregivers of nonverbal children and adults who are often unable to report troublesome medical issues in a timely manner. Individuals and caregivers also expressed the desire for treatments that did not suppress autistic traits, such as *stimming*, as a treatment consideration. Participants characterized treatment approaches targeting repetitive behaviors as underscoring many of the complex issues individuals with autism experience. Several participants expressed a need for treatments that “work with [autistic] traits rather than against.” Participants stressed the need for further research and understanding of the health effects of autism.

The patient input generated through this Patient-Focused Drug Development meeting and the public docket comments strengthens FDA's understanding of the impact of autism on individuals and their families and the therapies currently used to manage the effects of autism and co-occurring conditions. FDA staff will carefully consider this input during the drug development process, including when advising sponsors on their drug development programs and when assessing products under review for marketing approval. For example, Appendix 4 shows how this input may directly support our benefit-risk assessments for medical products under review. This input may also be of value to the drug development process more broadly. For example, it may be useful to drug developers as they explore potential areas of unmet medical need for autism. It could also point to the potential need for development and qualification of new outcome measures in clinical trials.

Topic 1: Disease Symptoms and Daily Impacts That Matter Most to Patients

The first discussion topic focused on individuals' and family members' perspectives on autism and how it impacts them and/or their loved ones' daily lives. Six panelists, two individuals with autism and four parents of children with autism, provided comments to start the dialogue.

The panelists are described further below:

- A 27-year-old woman, who shared her perspectives on holistic approaches to autism and her experiences with depression, anxiety, and suicidal ideation.
- A young adult woman, who shared her view on quality of life improvement and appropriate support for autism.
- The father of a 13-year-old, who described his son's experiences with complex communication difficulties.
- The mother of a 24-year-old, who shared her son's issues with mood disorders and sleep, as well as poor memory.
- The mother of three children with autism, a daughter (age 24) and two sons (age 22 & 19), highlighted the impact of autism on her children's overall health.
- The mother of a 5-year-old, who described her daughter's experiences with severe anxiety, sleep issues, and socially awkward behavior.

The panelists' statements provided a range of insight on daily impacts of autism. In the large-group facilitated discussion that followed the panel discussion, nearly all individuals with autism and caregivers indicated by a show of hands that their own experiences (or those of their loved ones) were reflected in the panelists' comments.

Perspectives on the health effects autism

In a polling question (Appendix 3, q5), participants were asked to identify up to three health effects of autism that have had the most significant impact on their daily lives. Based on the polling results and large-group facilitated discussion, communication difficulties and behavioral issues were identified as significant health effects of autism. Throughout the discussion, meeting participants offered differing perspectives on whether and how health effects related to autism should be managed. Participants

highlighted that some health effects are positive and enhance their and/or their loved one's ability to engage and interact daily.

The large-group facilitated discussion provided insight on the health effects of autism and co-occurring conditions. The range of health effects discussed with both in-person and web participants are described further below. In-person and web participants attempted to distinguish health effects of autism versus co-occurring conditions throughout the discussion. Our summary below attempts to convey the complexities of autism raised during discussion, using the participants' own words.

Behavioral Issues

Behavioral aspects of autism were frequently mentioned by meeting participants. Meeting participants highlighted clear distinctions between different types of behaviors associated with autism compared to co-occurring conditions and their significance. During the meeting, behaviors ranging from unwanted and possibly self-injurious to beneficial and often calming were described in detail.

Some participants, particularly caregivers of young children and adults highlighted their experience and concern with violent or aggressive behaviors. One father described moving his son into residential placement at age 9 because of aggressive behavior. He characterized his son's actions as "extremely violent homicidal ideation" which was "always directed toward other people." Another caregiver stated her son was "extremely violent toward property, himself and others." Another father shared that "he has hospitalized [his son] two times in the last three years for problems related to his mood." Throughout the larger group discussion, participants appeared to share a common perspective that the term "irritability," often used to describe bothersome behaviors in autism, does not adequately capture their experiences. One parent explained, "Irritability is . . . underplaying what many parents are dealing with . . . it's terrifying for a parent to see." Another parent shared this sentiment stating, "[It is] challenging behavior . . . It's not trivial."

Meeting participants also described their experiences with self-injurious behaviors or suicidal ideation. FDA was interested in participants' input on these experiences. Participants made a distinction between self-injurious behaviors and suicidal ideation or suicide attempts, and stated that they often have different causes. One individual with autism shared, "From an internal perspective . . . [they are] really different. And they're not motivated by the same things." Participants also explained that harming themselves was often not the intent of behaviors that become self-injurious. One participant shared, "I'm usually not trying to hurt myself. It just sort of happens."

During the discussion, individuals with autism and caregivers also communicated the value of assessing the source of self-injurious behaviors. One father attributed his son's suicidal behavior to developing severe depression during adolescence. He stated that his son "had thoughts of wanting to hurt himself, his siblings, and me." Other participants suggested evaluating if the individual with autism is "reacting to something or trying to communicate something is wrong" in cases of self-injurious behaviors. The correlation between communication difficulties and behavioral issues is described further in the statements below.

- "[My son's] behavior, [involved] sometimes kicking or pushing . . . if he's not able to communicate or he's not being [understood]."
- "When [my daughter] was nonverbal . . . [she expressed frustration with] a scream, banging her head, or biting herself."

- “In my son . . . I do link his behavior difficulties with difficulties in communicating.”

In-person and web participants also highlighted their **perspectives on non-injurious repetitive behaviors, or *stimming***. Based on polling question (Appendix 3, q5), repetitive behaviors were indicated as one of the least challenging aspects of autism by in-person and web participants. Participants referred to their repetitive behavior as *stimming*, and stressed that not all *stimming* behaviors are bothersome and, in fact, may be calming or pleasant. *Stimming* behaviors described as hand-flapping, tapping, or pacing, were frequently distinguished from “compulsive body focused behaviors such as picking their skin or pulling hair.” Meeting participants described compulsive body focused behaviors as unpleasant or unwanted. FDA expressed interest regarding meeting participants calming experiences with *stimming* and repetitive behaviors. Meeting participants further described their perspectives of *stimming* below:

- “*Stimming* doesn’t cause us problems. It often helps us focus and interact with the world around us.”
- “When I hand flap it makes me really happy and also helps with a bunch of other stuff.”
- “[*Stimming*] doesn’t bother me. And when I ask [my son] about it, it doesn’t bother him.”

Some meeting participants, however, expressed concerns regarding social stigma due to *stimming* behaviors. One family member shared that her daughter will awkwardly “point at a person who she’s talking to, jump up and down and flap her hands when overly excited.” An individual with autism also stated that her *stimming* behaviors are perceived “a little weird” to other people. Throughout the discussion, caregivers highlighted the concern of *stimming* upon social interactions, particularly for children. One caregiver shared, “The repetitive behaviors absolutely matter . . . [my son’s] mannerisms make him look different. He uses a squeeze ball or beads to control his hands.” Another caregiver shared, “The social aspect is one of my biggest fears . . . will she be bullied for being quote, unquote different?”

Communication difficulties

Throughout the meeting, individuals with autism and family members described numerous communication challenges. In-person and web participants noted communication issues such as nonverbal expression, delays in speech, inability to fully articulate words, and changes in tone of voice. As highlighted previously, meeting participants acknowledged the correlation between communication difficulties and behavioral issues. Some family members noted changes in communication due to autism in the statements below:

- “As a toddler, [my daughter] could still speak some words, but her speaking voice became less and less clear.”
- “After losing all speech at 11 months, [my son] regained speech at age two, right after going off dairy.”

Family members also expressed **extreme difficulties in understanding the needs of loved ones** with minimal communication. One father stated, “When [my son] is highly agitated or in apparent pain it can be difficult to pinpoint a cause.” Caregivers also highlighted the challenge communication difficulties can pose in daily activities. One father expressed the daily challenge communication posed in determining if his 22-year-old son needs to use the restroom. He stated, “We don’t know if [my son]

really has to go to the bathroom. We want to make sure he doesn't have an accident." Caregivers noted that overcoming communication difficulties can sometimes require prompting and patience. One parent shared that when reinforcing calming behavior her daughter's communication improves. Another parent highlighted the use of sign language and displaying photos via a computer tablet to navigate communication issues.

Throughout large-group facilitated discussion, meeting participants described experiencing an impaired form of communication due to autism which only becomes better or worse due to underlying issues. Family members particularly highlighted experiencing their loved ones' communication ability fluctuate due to underlying issues as illustrated in the statements below.

- "When [my son] feels better he does start using words, but most of the time [impaired communication] that's what we have."
- "When [my brother] is in an up period and he feels more at ease in communicating."

Meeting participants also focused on difficulties in expressive communication. Individuals with autism explained that there is often discordance between the words expressed and what they intended to say. As one participant stated, "There's a gap between what I want to say and what actually comes out of my mouth. Often what I end up saying is sort of a compromise between the block and what is actually possible." Another individual shared, "I had a feeling I was communicating pretty easily, but I wasn't exactly saying . . . what I wanted to say."

FDA was interested in learning more about the features of communication difficulties in terms of intensity, severity, or frequency. Meeting participants expressed difficulty in delineating features of communication difficulties that were most bothersome and stressed that each aspect of communication is important. Meeting participants described unique differences and challenges present in autism and the need to consider variance between individuals with autism.

Other Effects

In addition to behavioral issues and communication difficulties, in-person and web participants described other health effects that have a significant impact on their daily lives:

- Many participants described their/their loved one's challenges with **cognitive difficulties**. Meeting participants stated that cognitive difficulties make it harder to complete tasks effectively and may lead to safety concerns. One caregiver illustrated this by sharing experiences of his son walking into oncoming traffic and remaining missing for hours because he got on the wrong bus. Another caregiver noted cognitive impairment manifest as "slow processing speed and poor working memory" in her son.
- Some participants described the impact of **social impairment**. Individuals with autism also shared moments of social isolation, with some stating that they engaged with others online or through writing due to the difficulty of engaging socially in person. An individual with autism shared, "People who know me well know me online because there I can be my full self." One caregiver shared lack of social skills as one of her biggest fears for her daughter. Another caregiver expressed, to the contrary, that her 30-year-old son is very socially oriented and enjoys the company of people.

- A few participants noted that they or their child had a need for routines and **difficulty adjusting to changes in routines** or transitioning from tasks. For example, one mother described that before leaving the park, she must ask her 5-year-old daughter how much time she needs and set an alarm to ensure a smooth transition. Another caregiver expressed that his son needs “a lot of prompting to engage in or complete tasks.”
- A few participants also highlighted the impact of **restricted interest**. One meeting participant, with an autistic son and brother, stated that restricted interest prevents her brother from traveling “more than 20 miles from his house” and causes her son to become anxious when unable to occupy himself. An individual with autism also described this feeling as not being able to “control a lot [of] what I’m focusing on.” Another caregiver highlighted his son restricted interest as singing. Other caregivers noted fixation on specific objects, movies, and television shows as restricted interest their loved ones experience.
- Additional effects of autism mentioned throughout the meeting included issues with **sleep, gastrointestinal symptoms, allergies, and extreme overactivity**. Sleep issues mentioned included difficulties with falling asleep and staying asleep throughout the night. Gastrointestinal issues mentioned included diarrhea and constipation. Food and environmental allergies mentioned by participants included sensitivity to artificial dye and gluten. Extreme over activity was described as periods of uncontrollable episodes of laughing or crying.

Co-occurring Conditions

Throughout the discussion, meeting participants highlighted the presence of other co-occurring conditions that can impact their experiences with autism. Several meeting participants identified anxiety and depression as co-occurring conditions that often impact their experiences with autism. Many meeting participants expressed that communication and social interactions become increasingly difficult during moments of anxiety. One caregiver shared that his son is “more apt to be less communicative if he’s presenting as less anxious.” An individual with autism stated that when she is less depressed and anxious it is “a lot easier to leave the house. Do things like go to school or hold down a job.” Many individuals with autism indicated that health effects of autism remain constant while only co-occurring conditions change. One individual shared, “I don’t feel like my autism gets better or worse. But my anxiety does get better or worse.” Meeting participants also noted that some behaviors may be due to feelings of anxiety or depression. Throughout the discussion individuals with autism described depression and anxiety as quality of life issues overall. A few meeting participants also described experiences with additional co-occurring conditions such as attention deficit hyperactivity disorder (ADHD), obsessive compulsive disorder (OCD), and epilepsy.

Overall impact of autism on daily life

Throughout the meeting, in-person and web participants described the larger impact that autism has on their daily life, including:

- **The inability to engage socially and in public settings.** Individuals with autism and caregivers highlighted the social isolation they experience due to aggressive and disruptive behaviors. One caregiver shared that due to her daughter’s aggressive behavior, “We can’t go anywhere . . . [we were] not invited over to the neighbor’s barbeque.” Another caregiver shared fears of

crowds and large groups as a limitation in engaging socially. She stated, “[My daughter] couldn’t even approach the store without screaming.” One caregiver stated that communication difficulties have led his son to “isolating himself more and engaging less in social interactions.”

- **The impact on work and careers.** Meeting participants reported difficulty securing or sustaining employment due to autism. A few participants attributed difficulties in finding work to cognitive difficulties such as poor working memory. One individual with autism shared an experience of being fired for being “a bad cultural fit” and attributed the experience to doing “something socially inappropriate without even realizing it.”
- **The ability to live independently.** Meeting participants highlighted significant difficulties in performing daily activities due to autism, which limit their or their child’s ability to live independently. One caregiver expressed that every day he must assist his son in daily hygiene and basic life skills. He shared this aspect as “frustrating for [my son] because he becomes very reliant on us and I think he would like to have more independence than he can achieve at this point.” Several individuals with autism who participated in the meeting said that they do live on their own but require assistance in some areas. One individual with autism explained that she still depends on her mom to help pay bills and make phone calls.
- **The impact of social stigma.** Participants described the role of social stigma and the perception that autism traits are problematic. One individual stated, “The fact that something is a trait of autism doesn’t mean it is a problem of someone’s life.” Participants, particularly individuals with autism, shared how this perception affects their interactions socially and within the workplace. Family members expressed concern and fear for loved ones, particularly children, and the likelihood of future bullying due to perceived differences in behavior and communication. One caregiver shared that autistic traits, “make her son look different, and other kids at school see him different. All he wants is to be like other kids.”
- **The impact on family.** Caregivers also highlighted the drain of managing aggressive behaviors among families, particularly those with more than one child. One caregiver shared, “This sort of pattern [of aggressive behavior] . . . affects his younger sister when she gets caught up in it.” Another caregiver described excluding her son from a family vacation due to aggressive behavior. Another caregiver shared, “Our family’s focus is forced to center around dealing with that person’s increase in aggression or disruptive behaviors.”

Topic 2: Patient Perspectives on Treatments for Autism

The second discussion topic focused on experiences with therapies used to manage the effects of autism and co-occurring conditions. Three panelists (Appendix 2), an individual with autism and two parents of children with autism, provided comments to start the dialogue. The panelists shared their experiences, not only in the treatments used, but also on the factors that are considered when making decisions about treatments. Participants described the trial and error process of finding an effective therapy to manage their co-occurring conditions associated with autism. Panelists included:

- A young adult woman with autism who highlighted the need for treatments that address co-occurring conditions and preserve autistic traits.
- The father of a 30-year-old, who described his son’s treatment experiences with therapy to manage aggression, sleep, and seizure issues.
- The mother of a 10-year-old, who described her son’s treatment experiences with supportive therapies, dietary changes, and supplements to manage behavioral issues.

In the large-group facilitated discussion that followed, caregivers and individuals with autism indicated by a show of hands that their experiences (or those of loved ones) were reflected in the panelists’ comments. The facilitated discussion included prescription and over-the-counter therapies and a variety of non-drug treatments. In-person and web participants’ perspectives on treatment and considerations regarding treatment decisions are summarized below. This section concludes with a discussion of what participants would look for in an ideal treatment.

Perspectives on current treatments

Throughout the meeting, caregivers and individuals with autism highlighted the complexity of managing autism and selecting appropriate therapies. Meeting participants stressed the delineation between seeking treatment for co-occurring conditions and managing autism. Participants reported using a wide range of therapies, often in combination, to address co-occurring conditions, particularly anxiety, depression, and sleep issues. In the sections below, this report strives to convey the balance in management of autism versus that of co-occurring conditions as expressed by meeting participants.

Prescription and over-the-counter drugs

Based on polling question (Appendix 3, q7), participants were asked to identify types of therapies used to manage autism. In-person and web participants reported use of prescription and over the counter drugs, with varying degrees of effectiveness in managing their autism and co-occurring conditions. Meeting participants particularly highlighted depression, anxiety, and insomnia as conditions they intended to manage with prescription and over the counter drugs. During the large-group facilitated discussion, meeting participants indicated by show of hands that use of medical treatment is needed for some aspects of autism or co-occurring conditions. Participants also identified some of the specific co-occurring conditions they intended to manage with prescription and over the counter drugs. Meeting participants mainly focused on their treatment experiences with prescription drugs, such as antipsychotics, antidepressants, as well as over-the-counter drugs.

- Several participants described their experiences with the use of **antipsychotics**. Meeting participants highlighted the trial and error process of finding an appropriate antipsychotic medication for themselves or their loved one. One caregiver shared, “At one point [my son] was on five different psychoactive medications at the same time. Now he is fairly stable on two.” A young adult with autism shared, “I’ve been taking different psychiatric medications since high school.” Throughout the meeting use of antipsychotics was attributed to attempts to address issues with mood and aggression.
- Several participants described their experiences with the use of **antidepressants**. Meeting participants highlighted positive impacts of antidepressant use. One caregiver described the use of antidepressants in his son as making a “huge difference.” Another caregiver

attributed his son's medication regimen consisting of antidepressants as helping his son "to reduce overall incidence of self-injurious behaviors and aggression." Throughout the meeting, use of antidepressants was attributed to addressing issues with depression, anxiety, or mood disorders.

- A few participants shared experiences of using **other prescription drugs and over the counter products**. Other prescription drugs reported by participants included anticonvulsants and stimulants. Use of stimulants was described by meeting participants as helpful in addressing issues with attention and focus. One caregiver shared that her son "jumped 10 reading levels in one month," after beginning a stimulant that increased his focus. One caregiver highlighted the use of anticonvulsants in his son to prevent seizures. Participants also reported the use of over the counter and prescription sleep aids. Sleep aids were described as helpful in staying and falling asleep. Another caregiver stated, "[Sleep aids] have helped my son get four or more hours of sleep on most nights."

Non-drug Therapies

Participants emphasized the importance of non-drug therapies in the management of autism and associated co-occurring conditions. Specific non-drug therapies mentioned are included below.

- Several meeting participants described their experiences with using various types of **therapy** to manage autism. Types of therapy mentioned by meeting participants included speech therapy, occupational therapy, physical therapy, and behavioral therapy. Several meeting participants highlighted the positive impact of therapy for them or their loved ones. One parent stated, "Speech therapy has increased [my son's] expressive language." A few caregivers described the use of art and music therapy. One caregiver stated, "I am finding [music] therapy helpful for my son's speech and he loves it." Another caregiver also mentioned utilizing auditory processing therapy to assist her child in speaking in a normal tone. Participants also highlighted the difficulty in finding healthcare providers familiar with autism. One mother described finding a psychiatrist competent in mental health and development disability issues as "basically impossible."
- A few meeting participants mentioned the use of **diet modification** in managing autism. Diet modifications utilized by meeting participants included avoiding gluten and dairy. One caregiver shared that her son regained speech following removal of dairy from his diet. Meeting participants also mentioned the use of dietary supplements. Dietary supplements mentioned by meeting participants included folic acids, probiotics and vitamin B12. Meeting participants also mentioned sensitivity to food dye and artificial coloring which also impact their and/or their loved ones' diet.
- A few family members mentioned the use of **speech generating devices**, particularly for individuals who are nonverbal individuals. Their comments primarily centered on their loved ones' difficulty with utilizing devices due to other health impacts. One parent commented that her children were unable to use the device due to issues with self-control. Another caregiver stated, "[My son] can't use communication devices. He has them, just something [inside] won't let him."

- A few meeting participants commented on the **value of headphones** to control noise and sensory overstimulation. One individual with autism highlighted cost as a deterrent for use of noise canceling headphone more broadly in the autistic community.

Treatment downsides

Meeting participants described experiencing a number of downsides to their treatment approaches. Meeting participants highlighted the uncertainty of initiating new treatments due to lack of research regarding medication response in autistic individuals. For example, one individual with autism stated, “Anecdotally autistic people seem to be more sensitive and respond atypically to many medications.” Other participants, noted their uncertainty lies in an inability to pinpoint which aspect of autism a medication will treat. One participant described this process as, “stripping away the layers of an onion.” Several participants described the challenges of pursuing a treatment to manage one aspect of autism, while leaving other areas of autism unaddressed as “frustrating.”

In-person and web participants also emphasized the significant impact of treatment side effects, especially when using medications long-term. Some participants expressed concern in initiating or continuing treatments because of likelihood of side effects or decreased benefit over time. Caregivers, particularly of nonverbal children, expressed concerns for loved ones who may experience side effect that cannot be communicated in a timely manner. One caregiver shared that her nephew experienced “a state of paralysis” due to a medication side effect which required physical and occupational therapy. One individual with autism shared developing akathisia, an uncontrollable movement disorder, from the use of antipsychotics. Participants also described experiencing other side effects such as weight gain, increased aggression, cardiovascular issues, and constipation.

Meeting participants also highlighted how medication formulation and drug monitoring can impact their decision-making process. One caregiver stated, “[My son] cannot tolerate the laboratory blood draws necessary to monitor him properly” which caused her son to eventually “abandon” a particular treatment. Participants also highlighted the difficulties of finding medications that are free of food dyes, artificial colors, and in easy to administer dosage forms. One caregiver shared, “[My son] was so sensitive to food dyes and he wasn’t able to swallow pills, we had a lot of our meds compounded into a liquid form.” Another caregiver noted the pill burden associated with managing autism. She highlighted that her son must take “a handful of pills everyday” along with a B12 shot every 4 days.

A few participants also commented on the cost of treatment, including out-of-pocket cost for treatments not covered by insurance. One participant stated, “We spend about 20 hours a week in therapy and about \$20,000 a year devoted to his treatments.”

Perspectives on an ideal treatment

Participants provided feedback on what they would look for in an ideal treatment for autism. During discussion of ideal treatments, individuals with autism stressed that their treatment needs and goals often differ from their family members. One individual with autism shared, “We are autonomous people from our parents and often have different goals and needs than they do.” A parent also highlighted this by sharing her son’s statement: “Mom, you know I disagree with you and the doctors about autism and bipolar. I do not like those labels. I am just a quirky, oddball kind of guy who likes to do things his own

way.” Throughout the discussion FDA attempted to both acknowledge and capture these variations in perspectives further described below.

Meeting participants, particularly individuals with autism, highlighted the importance of developing treatments that improve quality of life for individuals with autism. Throughout the meeting participants stressed the differences between treating autism versus co-occurring conditions. Individuals with autism stressed that an ideal treatment should work with autistic traits. As one participant explained, “Therapies intended to suppress autistic traits hurt autistic people.” Individuals with autism stressed that treatments that target nonharmful repetitive behaviors, or stimming, are counterproductive, because those behaviors are useful to individuals with autism as mechanisms to cope with stress or sensory over stimulation. Caregivers stressed the need for medication dosage forms that are more accommodating to sensitivities present in autistic individuals. A few in-person and web participants noted that an ideal treatment should be affordable, free from artificial coloring or flavors, and available in liquid dosage forms.

Throughout the discussion, meeting participants stressed that ideal treatment begins by enhancing the medical community’s understanding of autism and its co-occurring conditions. Participants commented that individuals with autism need greater representation in drug development programs and overall support to improve quality of life. Participants also commented on the need to understand how individuals with autism experience symptoms associated with other co-occurring conditions.

Summary of Comments Submitted to the Public Docket

Forty-one comments were submitted to the public docket that supplemented the Patient-Focused Drug Development public meeting on autism. The majority of comments were submitted by family members and individuals with autism. Five comments were submitted by advocacy organizations, including Clearly Present Foundation, Autistic Self Advocacy Network, Advocates for Autism of Massachusetts, Association of University Centers on Disabilities and Autism Speaks. One survey was submitted by a special needs day and residential school.

Overall, the docket comments reflected the experiences and perspectives shared at the May 2017 meeting. The comments further elaborate on several issues raised during the meeting. The following section provides highlights on daily impacts of autism, management of autism, treatment of co-occurring conditions, and perspectives on an ideal treatment. This section particularly focuses on experiences or perspectives that were not raised or addressed in detail at the meeting.

Submitted comments on the effects of autism

The docket comments reiterated the health effects of autism and co-occurring conditions in lives of individuals with autism and their family members. The health effects described were generally consistent with those discussed at the meeting and included communication difficulties, behavioral issues, and co-occurring conditions such as depression and anxiety. General comments from autism advocacy organizations also generally aligned with meeting input.

Similar to in-person and web participants, docket commenters highlighted the significant impact of communication difficulties and behavioral issues. A few commenters also described how communication difficulties have changed over time. For example, a grandparent described his granddaughter’s

regression in speech beginning at two years of age, which led her to become nonverbal. Commenters particularly focused on how communication difficulties may lead to aggressive behavior. Commenters shared their experiences of aggressive, repetitive, restricted, and self-injurious behaviors, such as kicking, use of threatening language, punching, and biting. Similar to meeting participants, docket comments also described the benefits of stimming behaviors in self-regulation during moments of overstimulation or anxiety.

Other health effects mentioned in the docket included gastrointestinal issues, sleep issues, social issues, and cognitive difficulties, summarized below:

- Several docket comments described the difficulty of social interactions, which they described as awkward and demanding. One 34-year-old with autism shared that childhood bullying and social rejection throughout life has left her “wary” of seeking social interactions. One parent stated that her son, “cannot participate safely in social activities” due to anxiety and aggressive behaviors. Another parent shared socializing is difficult due to her daughter’s “meltdowns” in group settings or public spaces.
- Several docket comments highlighted issues with falling asleep or staying asleep. One commenter shared, “Our daughter woke up every 2–3 hours for the first two years of her life.” Caregivers also highlighted the impact of their child’s sleep issues on overall quality of life, particularly due to sleeping in the same bed as parents and awakening frequently during the night to care for children. One caregiver stated, “I would just sleep when [my daughter] slept . . . It was like having a newborn for 2.5 years.”
- A few docket comments addressed the impact of cognitive difficulties. Some highlighted the challenges faced by their loved ones when interacting with peers in school and keeping up with coursework. Individuals with autism highlighted the difficulty of cognitive impairment in the workplace. For example, one individual described having a “limited cognitive bandwidth” which makes it difficult for her to keep up with the multitasking demands of her job.
- A few docket comments touched upon gastrointestinal issues. One caregiver shared that her daughter’s symptoms became so severe she stopped eating for three days. Another caregiver highlighted her son’s issues with “terrible, painful constipation.” Throughout the docket some commenters noted gastrointestinal issues as due to medication side effects or changes in diet.
- A few docket comments also briefly highlighted issues with restricted interest. One caregiver described restricted interest in her son as being “highly focused only on playing with specific toys.” An individual with autism described restricted interest as inability to “control a lot [of] what I’m focusing on.” Another caregiver stated her son’s interest are so restrictive, “he starts getting anxious . . . and he doesn’t know how to occupy himself” if in the home for more than 30 minutes.
- A few docket comments described food sensitivities and dietary issues. One caregiver described his granddaughter’s food sensitivities stating she has “a limited list of foods she is willing to eat or drink.” Other docket commenters described experiences with food allergies, diet interventions, and use of dietary supplements to manage issues with food.

Submitted comments on the overall impact of autism on daily life

The docket comments reflected the input received during the meeting related to the impacts of autism on daily life. Most commenters highlighted the impact of autism on performing activities, maintaining and building interpersonal relationships and impacts on work. Further insight provided into impacts on daily life are described below.

- Commenters also shared impacts of autism upon living independently. Several caregivers expressed concern regarding their child's ability to live independently. One caregiver shared, "What worries me most in the future is . . . [my son's] ability to live independently." Like in-person and webcast participants, docket commenters highlighted how the inability to perform daily activities leads to this concern. For example, one caregiver described getting dressed or brushing teeth as "impossible tasks" for her son. Another caregiver shared his granddaughter is unable to feed herself independently."
- Commenters also highlighted the impact on work and careers. One individual with autism shared, "I have fears related to continuing to be able to advance my career and also take care of myself." Another individual highlighted the emotional toll of not being able to find work: "If I had a full-time job then I would feel like I was contributing more to society and wouldn't feel like such a burden." A family member also shared her autistic cousin's financial difficulty due to inability in finding full-time employment and only working "several low-paying jobs." Many docket commenters also noted discrimination and lack of workplace accommodations as a barrier to finding work.
- Commenters reiterated the impact of autism and behavioral issues on interpersonal relationships. One caregiver highlighted the difficulty of placing his son in a residential treatment at 9 years of age because of the "physical and emotional threat he represented to his siblings." Family members described feeling "trapped and afraid" due to dealing with aggressive, disruptive, and self-injurious behaviors. One parent shared, "Our family lives with a level of tension every day, anticipating if [my son] is going to have a good day." Throughout the docket commenters often described a sense of isolation due to lack of ability to form interpersonal relationships.

Submitted comments on current treatments for autism

The submitted comments regarding current treatment options and experiences with autism and co-occurring conditions were similar to those expressed at the public meeting. Use of antipsychotics, antidepressants, anticonvulsants, and sleep aids were commonly discussed by docket commenters. Similar to in-person participants docket comments described a trial and error process to finding a suitable medication regimen. One caregiver stated that finding a medication regimen that worked "took two years and a lot of misery to find." Another caregiver shared that "only through trial and error over 13 years that a reasonably acceptable cocktail of medication has been identified." Similar to in-person participants, commenters shared their and/or their loved ones' experiences with managing a wide range of co-occurring conditions including depression, anxiety, behavioral issues, sleep issues, epilepsy, obsessive compulsive disorders, and attention deficit hyperactivity disorder.

Similar to the participants in the public meeting, many docket commenters noted their treatment regimen resulted in a range of side effects, some of which led to discontinuation of treatment. Side

effects mentioned in the docket included weight gain, gastrointestinal issues, and increased aggressive behavior. Comments also noted shifts in personality that occurred following medical treatment and the desire to balance treating co-occurring conditions and managing autism. One caregiver explained that although his son's medication assisted with aggression, "He is no longer the happy kid he used to be." Another caregiver shared that her son's medication "impacts his social skills, makes him anxious and distant."

A range of nondrug therapies were also mentioned in the docket comments. These included diet modifications, therapy (behavioral, occupational, physical, and psychotherapy), medical devices, and noise cancellation headphones. Like in-person and web participants, commenters highlighted using therapy and prescription medication together to improve health effects of autism. Docket commenters also noted the time constraints on therapy for autism and co-occurring conditions, given other family activities. She shared, "Family meal time is usually delayed due to ABA (applied behavior analysis) which leaves some [family] members feeling hungry and angry."

Individuals with autism and caregivers also expressed difficulty with finding practitioners skilled in providing care and appropriate therapy for autism. A few participants also commented on the cost of treatment. One caregiver with two autistic children estimated \$50,000 per year in out-of-pocket cost for management of autism including medication, therapy, and dietary requirements.

Submitted comments on ideal treatments for autism

Several perspectives were provided on ideal treatments for autism and co-occurring conditions. Several comments expressed the need for continued research on autism to better understand the medical needs of autistic individuals. Similar to in-person participants, docket commenters also desire medical interventions that preserve autistic traits. An individual with autism stated, "I'm interested in medications that will allow us to alleviate our discomforts rather than . . . take away our unique gifts." A caregiver also expressed, "[My son] would object any intervention that would impact his personality." Commenters also noted an ideal treatment would be affordable, have less side effects, and available in an easy to administer dosage form.

Conclusion

FDA recognizes that individuals with autism and their caregivers have a unique ability to contribute to our understanding and that of others involved the drug development process. The input provided during this meeting highlights the need for additional research, clinical endpoints and improved therapies in autism management and treatment. This meeting underscored the diverse range of perspectives within the autism community. This Patient-Focused Drug Development meeting enabled FDA to gain insight on not only the health effects and impacts of autism but also the challenges the autistic community faces with co-occurring conditions such as depression, insomnia, and anxiety. Particularly, the agency gained insight into the complex communication, behavioral, and social issues that significantly impact the daily life of individuals with autism.

We greatly appreciate the openness of individuals with autism and their caregivers, who courageously and candidly shared their experiences and perspectives through the Patient-Focused Drug Development meeting and docket. FDA shares the autism community's desire and commitment to furthering the development of new safe and effective drug therapies for autism and co-occurring conditions.

Appendix 1: Meeting Agenda and Discussion Questions

Public Meeting on Patient-Focused Drug Development for Autism

May 4, 2017

12:00 – 1:00 pm	Registration
1:00 – 1:05 pm	Welcome Sara Eggers, PhD <i>Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA</i>
1:05 – 1:10 pm	Opening Remarks Ellis Unger, MD <i>Director, Office of Drug Evaluation 1 (ODE 1), Office of New Drugs (OND), CDER, FDA</i>
1:10 – 1:20 pm	Overview of FDA’s Patient-Focused Drug Development Initiative Pujita Vaidya, MPH <i>OSP, CDER, FDA</i>
1:20 – 1:30 pm	Overview of Autism and Current Treatment Options Tiffany Farchione, MD <i>Deputy Director, Division of Psychiatry Products (DPP), OND, CDER, FDA</i>
1:30 – 1:35 pm	The Road from PFDD Meetings to Clinical Trial Endpoints Ebony Dashiell-Aje, PhD <i>Clinical Outcome Assessments Staff, OND, CDER, FDA</i>
1:35 – 1:40 pm	Overview of Discussion Format Sara Eggers, PhD <i>OSP, CDER, FDA</i>
1:40 – 2:00 pm	Panel #1 Discussion on Topic 1: Health Effects and Daily Impacts of Autism Topic 1: A panel of individuals and caregivers will provide comments to start the discussion on significant health effects and daily impacts of autism.
2:00 – 3:00 pm	Large-Group Facilitated Discussion: Topic 1 Individuals and caregivers in the audience will be invited to add to the dialogue.
3:00 – 3:10 pm	Break
3:10 – 3:30 pm	Panel #2 Discussion on Topic 2: Current Approaches to Treatment Topic 2: A panel of individuals and caregivers will provide comments to start the discussion on current approaches to treating autism.
3:30 – 4:25 pm	Large-Group Facilitated Discussion: Topic 2 Individuals and caregivers in the audience will be invited to add to the dialogue.
4:25 – 4:55 pm	Open Public Comment
4:55 – 5:00 pm	Closing Remarks Mitchell Mathis, MD <i>Director, DPP, OND, CDER, FDA</i>

Docket Information: We encourage you to submit your written comments to the docket by July 5, 2017: <https://www.federalregister.gov/d/2017-04229> or go to www.regulations.gov and search for: **autism patient-focused drug development**.

Appendix 2: Patient and FDA Panel Participants

Patient Panel, Topic 1

- Nadine Morris
- Zoe Gross
- Sharrill Hemry
- Tom Frazier
- Sara Luterman
- Kiely Law

Patient Panel, Topic 2

- Kathleen (Kit) Mead
- Susan Pannell
- Tom Hubbard

FDA Panel

- Ellis Unger, Office of Drug Evaluation 1 (ODE 1), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER)
- Mitchell Mathis, Division of Psychiatry Products (DPP), OND, CDER
- Tiffany Farchione, DPP, OND, CDER
- Juliette Toure, DPP, OND, CDER
- Ebony Dashiell-Aje, Clinical Outcomes Assessment (COA), OND, CDER
- Kerry Jo Lee, Division of Gastroenterology and Inborn Errors Products (DGIEP), CDER

Appendix 3: Meeting Polling and Scenario Questions

The following questions were posed to in-person and web meeting participants at various points throughout the May 4, 2017, Patient-Focused Drug Development for Autism public meeting. Participation in the polling questions was voluntary. The results were used as a discussion aid only and should not be considered scientific data.

Demographic Questions

1. Where do you live?
 - a. Within Washington, D.C. metropolitan area
 - b. Outside of the Washington, D.C. metropolitan area
2. I am:
 - a. A self-advocate, an individual with autism
 - b. A family member of an individual(s) with autism
3. Do you/your loved one with autism identify as:
 - a. Male
 - b. Female
 - c. Other
4. What is your/your loved one's age?
 - a. Younger than 5
 - b. 5 – 12
 - c. 13 – 17
 - d. 18 – 29
 - e. 30 – 39
 - f. 40 – 49
 - g. 50 – 59
 - h. 60 or greater

Question for Topic 1

5. Which health effects of autism are most challenging to you/your loved one? **Please choose up to three health effects.**
 - a. Irritability or disruptive behaviors
 - b. Cognitive impairment
 - c. Social impairments
 - d. Communication difficulties
 - e. Repetitive behaviors
 - f. Sleep issues
 - g. Depression or anxiety
 - h. Gastrointestinal symptoms
 - i. Other symptoms not mentioned

6. What aspects of your/your loved one's daily life are most negatively affected by autism? **Please choose up to three aspects.**
- a. Ability to participate in or perform daily activities (such as work, school, sports, drive, hobbies, etc.)
 - b. Ability to care for self or family
 - c. Risks to safety of self or others
 - d. Impact on relationships with friends and family
 - e. Stigma or social discrimination
 - f. Emotional impacts (such as fear, embarrassment, self-esteem, etc.)
 - g. Burden of medical care
 - h. Other impacts not mentioned

Question for Topic 2

7. Have you/your loved one ever used any of the following therapies to help reduce symptoms of autism? **Check all that apply.**
- a. Prescription medications (such as anticonvulsants or psychiatric medications)
 - b. Psychotherapy and behavioral therapy (such as counseling or support groups)
 - c. Speech therapy
 - d. Physical therapy
 - e. Occupational therapy
 - f. Diet modifications
 - g. Other therapies not mentioned
 - h. I've never used any therapies
8. When considering treatment options, which of the following benefits would you/your loved one consider to be most important? **Please choose up to three.**
- a. Reduced irritability and/or disruptive behaviors
 - b. Reduced cognitive impairment
 - c. Reduced social impairments
 - d. Reduced communication difficulties
 - e. Reduced repetitive behaviors
 - f. Reduced sleep issues
 - g. Reduced depression or anxiety
 - h. Reduced gastrointestinal symptoms
 - i. Other
9. When considering treatments, which ONE benefit would you/your loved one consider to be most meaningful? **Please choose one.**
- a. Reduced irritability and/or disruptive behaviors
 - b. Reduced cognitive impairment
 - c. Reduced social impairments
 - d. Reduced communication difficulties
 - e. Reduced repetitive behaviors
 - f. Reduced sleep issues
 - g. Reduced depression or anxiety
 - h. Reduced gastrointestinal symptoms
 - i. Other

SCENARIO:

Imagine that a new oral medication indicated to treat repetitive behaviors associated with autism has recently been approved by FDA. Your doctor believes that you/your loved one may be a good candidate for this medication.

The medication requires administration every six hours and evaluation every eight weeks for dosage adjustment. The tablet can be crushed and mixed with food for ease of administration.

Common side effects of this medication include fatigue, drowsiness, diarrhea, nausea and insomnia. Rare and severe side effects such as respiratory tract infections and blood clots are rare, but possible.

**What first thoughts come to mind as you hear this scenario?
What questions would you ask your doctor about this treatment?**

10. Which **TWO** would you rank as most important to your decisions about whether to use a medication to help manage autism symptoms?
- a. How the medication is administered
 - b. The frequency and length of treatment
 - c. Your access to treatment (such as insurance coverage)
 - d. Whether the medication was studied in children
 - e. How much the medication showed benefit for a specific symptom
 - f. Common side effects
 - g. Rare, but serious side effects
 - h. Your/your loved one's previous response to a similar treatment
 - i. Whether other treatment options are available

Appendix 4: Incorporating Patient Input Into a Benefit-Risk Assessment Framework for Autism

Introduction

Over the past several years, FDA has developed an enhanced structured approach to benefit-risk assessment in regulatory decision-making for human drugs and biologics³. The Benefit-Risk Assessment Framework involves assessing five key decision factors: *Analysis of Condition*, *Current Treatment Options*, *Benefit*, *Risk*, and *Risk Management*. When completed for a particular product, the Framework provides a succinct summary of each decision factor and explains FDA's rationale for its regulatory decision.

In the Framework, the *Analysis of Condition* and *Current Treatment Options* rows summarize and assess the severity of the condition and therapies available to treat the condition. The assessment provides an important context for drug regulatory decision-making, including valuable information for weighing the specific benefits and risks of a particular medical product under review.

The input provided by patients and patient representatives through the Patient-Focused Drug Development Public Meeting for Autism and docket comments will inform our understanding of the *Analysis of Condition* and *Current Treatment Options* for this disease.

The information in the top two rows of the sample framework for autism below draws from various sources, including what was discussed at the Patient-Focused Drug Development Public Meeting for Autism conducted on May 4, 2017. This sample framework contains the kind of information that we anticipate could be included in a framework completed for a drug under review for autism. This information is likely to be added to or changed over time based on a further understanding of the condition or changes in the treatment armamentarium.

³ Commitments in the fifth authorization of the Prescription Drug User Fee Act (PDUFA V) include further development and implementation of the Framework into FDA's review process. Section 905 of the FDA Safety and Innovation Act also requires FDA to implement a structured benefit-risk framework in the new drug approval process. For more information on FDA's benefit-risk efforts, refer to <http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>.

Dimensions	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition	<ul style="list-style-type: none"> • Autism is a complex neurodevelopmental disorder that can cause communication, behavioral, and social challenges. There is no medical test for diagnosis, autism is diagnosed based on behavior and developmental screening. • Experiences common in children and adults with autism include: trouble relating to others or lack of interest in people, extreme sensitivity to certain sounds, smells or lights, repetitive behavior pattern, having narrow obsessive interests, and difficulty adapting to changes in routines. • Individuals with autism have noted some repetitive behaviors as beneficial as they cope with sensory stimuli and anxiety. These behaviors, known as <i>stimming</i>, may include hand flapping, pacing, and tapping. • Individuals with autism may also experience co-occurring conditions such as depression, anxiety, insomnia or mood disorders. 	<p>Autism is a complex neurodevelopmental disorder. Effects of autism vary from person to person and can range from mild to severely debilitating. Cognitive, social, and behavioral issues associated with autism can significantly impact a patient's quality of life and may reduce likelihood of living independently and performing daily task without assistance.</p>
Current Treatment Options	<ul style="list-style-type: none"> • FDA approved therapies for autism are limited to treatments for irritability. Approved treatments include risperidone (for ages 5 to 16) and aripiprazole (for ages 6 to 17). • The mainstay of treatment is behavioral therapies and educational interventions. For children, behavioral therapies in the form of the early intervention programs often emphasize play-based interactions that encourage shared attention and communication. For adults, services are more vocational, home and community based such as social skills workshops and support groups. 	<p>There is currently no cure for autism. Currently, drugs are approved for treatment of irritability associated with autism; however, irritability may not fully characterize many of the health effects of autism which matter most to individuals and their families.</p> <p>No drug therapies currently target the underlying causes or core manifestations of autism.</p>