Biomedical Therapy for Autism

Our easy-to-read fact sheets provide clinicians with reliable information to share with patients and their caregivers.

Biomedical therapy for autism spectrum disorder (ASD) is becoming increasingly popular as a complementary treatment option to traditional medication regimes, yet many patients are still unclear about what biomedical therapy entails. Therefore, the following fact sheet provides a helpful overview of biomedical therapy for ASD and answers commonly asked questions.

Autism Spectrum Disorder

Autism spectrum disorder (ASD) is a neurodevelopmental disorder associated with impairment in social communication and interactions as well as the presence of restricted, repetitive behaviors.\(^1\) It is influenced by both genetic and environmental factors, though the direct cause is still unknown.\(^2\)

The Diagnostic and Statistical Manual of Mental Disorders, Fifth Edition, Text Revision (DSM-5-TR) is considered the gold standard for ASD diagnosis. The diagnostic criteria for ASD are graded on a severity scale by the level of support needed, in which Level 1 requires support, Level 2 requires substantial support, and Level 3 requires very substantial support.\(^2\)

Common Treatment Options for Autism

Because ASD occurs on a spectrum, treatment options can vary with each patient depending on their unique set of symptoms. There is no curative treatment for ASD, but the management of ASD takes on a multifaceted approach that includes occupational, behavioral, speech, and play therapies. Psychosocial interventions can also help improve specific behaviors, such as language and social engagement.\(^3\)

Although there are no medications directly indicated for the treatment of ASD specifically, many individuals receive medication for comorbidities associated with their diagnosis. Patients with ASD may take medication for irritability, aggression, hyperactivity, and seizures that may co-present with the condition.\(^2\) Some examples of the common medications used to manage these other symptoms and disorders may include:\(^4\)
• Psychostimulants, such as **methylphenidate** and **dextroamphetamine**
• Antidepressants, such as **sertraline** and **fluoxetine**
• Antipsychotic medications, such as **risperidone** and **aripiprazole**
• Antiepileptic medications, such as **carbamazepine**, **levetiracetam**, and ** ethosuximide**
• Alpha-2 adrenergic receptor agonists, such as **clonidine** and **guanfacine**

Biomedical Therapy for Autism

Biomedical therapy is a specific treatment approach that considers the underlying biological basis of a condition and targets physiological impairment.\(^5\) The goal of biomedical therapy for ASD is to optimize physiological factors impacting brain function and development to treat symptoms and improve patient functioning.

Research indicates that ASD is associated with deficits in mitochondrial metabolism and oxidative stress as well as abnormalities in the regulation of the following essential metabolites:\(^6\)

- Folate
- Tetrahydrobiopterin
- Glutathione
- Cholesterol
- Carnitine
- Branch chain amino acids

Biomedical therapy can be categorized based on the pathophysiological process they target:

**Mitochondrial dysfunction**
The mitochondria generate energy for cellular processes. When the mitochondria is impaired, it can lead to developmental delays, muscle weakness, and neurological problems.\(^6\) Individuals with ASD who have mitochondrial dysfunction often have more severe behavioral and cognitive deficits, relative to those with typical mitochondrial function. Treatments may include:\(^5\)

- Antioxidants, such as vitamin C and N-acetyl-L-cysteine
- L-carnitine
- Multivitamins containing vitamin B, vitamin E, co-enzyme Q10
Folate Metabolism
Folate is naturally found in the human body and helps to regulate the absorption of vitamin B. However, individuals with ASD may have genetic modifications in the folate pathway which leads to a decrease in available folate in the brain, known as cerebral folate deficiency. Lack of folate causes symptoms such as fatigue and muscle weakness. Patients with folate irregularity are treated with folinic acid for neurological, behavioral, and cognitive improvements.6

Redox Metabolism
Redox reactions are necessary for many biological functions. Evidence has shown that individuals with ASD may have abnormal redox metabolism which could lead to oxidative damage in areas of the brain responsible for speech, emotion, and social behavior. Several treatments for oxidative stress are available, including:6

- Vitamin or mineral supplements containing antioxidants, co-enzyme Q10, and vitamin B
- Subcutaneous injections of methylcobalamin (a form of vitamin B12)
- Oral folinic acid
- Tetrahydrobiopterin supplementation
- N-acetyl-L-cysteine

These treatment options can help improve many common ASD symptoms, including hyperactivity, tantrums, sensory-motor skills, irritability, and even sleep and gastrointestinal symptoms.

Tetrahydrobiopterin Metabolism
Tetrahydrobiopterin (BH4) is naturally found in the body and is necessary for multiple important metabolic pathways. Abnormalities in BH4 are prominent in ASD, as the disorder is associated with a lack of oxidative stress needed for BH4 pathways. Treatment for BH4 metabolic dysfunction is primarily the use of sapropterin, a synthetic form of BH4. Sapropterin has been shown to improve cognitive ability, communication, adaptability, verbal expression, and social function in patients with ASD.6

Frequently Asked Patient Questions

At what stage should I consider biomedical treatment?
Because ASD is a lifelong condition that occurs on a spectrum, there is no standard timeline for when a treatment should be started.7 Interventions are tailored to the patient’s specific needs. Although supplements are generally
safe and well tolerated, they can have interactions with certain medications. Speak with your healthcare provider when making decisions on treatment options as it is important to keep track of your medications to monitor improvement and prevent adverse medication interactions.

How long does it take to see improvement?
When evaluating treatment success, it is important to consider what symptoms or conditions are being targeted. For example, some patients may be seeking treatment to sleep better or decrease their repetitive behaviors. Tracking progress by logging symptoms and improvements can help you and your provider gain an accurate measure of your treatment response. Improvements may not be seen immediately, especially as certain medications can take time to show measurable effects. Your symptoms may fluctuate over time, so consistent medication adherence is necessary to improve your chances of overall improvement.8

Are there side effects associated with these treatments?
All of the biomedical treatments mentioned throughout this fact sheet are generally considered safe and well-tolerated. However, there are minor side effects for some of these treatments, as detailed below:
- Patients taking L-carnitine have expressed slight gastrointestinal issues. Symptoms such as nausea, vomiting, and abdominal cramps are usually experienced when the supplement is taken at night on an empty stomach. To minimize these symptoms, these supplements can be taken after a meal and your time of dosing can be adjusted.6
- Some patients taking high-dose folinic acid may experience increased irritability, insomnia, or gastroesophageal reflux when co-administered with other medications, such as antipsychotics.6
- Individuals taking N-acetyl-L-cysteine may experience mild side effects such as constipation, fatigue, daytime drowsiness, or increased appetite.6

Not all patients respond to treatment options in the same way. Patients should speak with their providers to discuss their treatment plan and any potential side effects they may experience.