Understanding Stem Cell Therapy for Autism Spectrum Disorder

A Patient Guide from Auragens

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Advancing Regenerative Medicine:
Treatments & Empathy Amplified by Science and Technology

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Welcome

Dear Families,

Thank you for taking the time to learn about investigational stem cell therapy for Autism Spectrum Disorder (ASD). We understand that navigating treatment options for your loved one can feel overwhelming, especially when considering emerging therapies that are still being studied.

At Auragens, we are committed to providing you with clear, evidence-based information about human umbilical cord-derived mesenchymal stromal cells (hUC-MSCs) from Wharton's Jelly—a promising investigational approach that is being explored in clinical research around the world. This guide is designed to help you understand what these cells are, how they may work in ASD, what the current research shows, and what to expect if you choose to explore this therapy.

Our goal is to empower you with knowledge so that you can make informed decisions in partnership with your healthcare team. We recognize that every individual with ASD is unique, and outcomes can vary. This therapy is investigational, meaning it is still being studied and is not a cure. However, early research has shown promise, and we are honored to walk alongside families seeking hope and new possibilities.

With compassion and respect,
The Auragens Team

1. Understanding Autism Spectrum Disorder and Current Treatment Limitations

Autism Spectrum Disorder (ASD) is a complex neurodevelopmental condition that affects how individuals communicate, interact socially, and experience the world around them. ASD is called a "spectrum" because it presents differently in each person—some individuals may have significant challenges with language and daily living skills, while others may have milder symptoms and excel in certain areas.

The exact causes of ASD are not fully understood, but research suggests that a combination of genetic, environmental, and biological factors contribute to its development. In recent years, scientists have discovered that immune system dysfunction and inflammation in the brain—often called neuroinflammation—may play an important role in ASD. Many children with ASD show signs of an overactive immune response, and studies have found elevated levels of inflammatory molecules in the brain and blood of individuals with autism.

Current Treatment Landscape

Today, the most widely used treatments for ASD focus on behavioral and educational interventions, such as Applied Behavior Analysis (ABA), speech therapy, occupational therapy, and social skills training. These therapies can be very helpful in improving communication, learning, and daily functioning. Some individuals also benefit from medications that address specific symptoms like anxiety, hyperactivity, or irritability.

However, these approaches do not address the underlying biological processes that may contribute to ASD, such as immune dysregulation and neuroinflammation. There is currently no medical treatment that targets the root causes of autism or reverses its core features. This gap has led researchers to explore new biological therapies, including stem cell treatments, that may help modulate the immune system and reduce inflammation in the brain.

Why Families Are Exploring Stem Cell Therapy

Families are increasingly interested in stem cell therapy because it represents a fundamentally different approach—one that aims to address some of the biological mechanisms thought to contribute to ASD. While stem cell therapy is still investigational and not a cure, early research has shown that it may help improve certain symptoms in some children by calming immune overactivity and supporting healthier brain function. This guide will help you understand how this therapy works, what the research shows, and what to expect if you choose to explore it for your loved one.

2. What Are hUC-MSCs from Wharton's Jelly?

Human umbilical cord-derived mesenchymal stromal cells, or **hUC-MSCs**, are a type of stem cell that comes from a special tissue inside the umbilical cord called **Wharton's Jelly**. The umbilical cord connects a developing baby to the mother during pregnancy, and after birth, it is typically discarded as medical waste. However, scientists have discovered that Wharton's Jelly contains a rich source of powerful cells that can be collected, processed, and used for medical therapies.

What Makes These Cells Special?

Mesenchymal stromal cells (MSCs) are not the same as embryonic stem cells. They are adult-type stem cells that have unique properties:

- **Immunomodulatory**: They can help regulate and calm an overactive immune system.
- **Anti-inflammatory**: They release molecules that reduce inflammation throughout the body, including in the brain.

- **Regenerative signaling**: They secrete growth factors, proteins, and tiny packages called exosomes that communicate with other cells and support tissue repair and healthy function.
- **Low immunogenicity**: Because they come from the umbilical cord, hUC-MSCs are considered "immune-privileged," meaning they are less likely to be rejected by the recipient's immune system compared to other types of transplanted cells.

How Are They Collected and Prepared?

After a healthy baby is born, the umbilical cord is donated by the mother with informed consent. The cord is processed in a specialized laboratory, where Wharton's Jelly is carefully extracted. The MSCs are then isolated, expanded (grown in controlled conditions to increase their number), tested for safety and quality, and cryopreserved (frozen) until they are ready to be used.

At Auragens, we use only hUC-MSCs that meet the highest standards of safety, purity, and potency, as verified by our **AABB accreditation** (more on this in Section 3).

How Are They Delivered?

In the clinical studies discussed in this guide, hUC-MSCs are typically delivered through **intravenous (IV) infusion**—a process similar to receiving fluids or medication through an IV line. The cells travel through the bloodstream and are thought to reach areas of the body where they are needed, including the brain, where they may help reduce inflammation and support healthier neural function.

🏆 **Why Auragens: AABB Accreditation — The Gold Standard in Cellular Therapy**

Auragens Stem Cell Center is proud to be AABB accredited — a critical distinction that sets us apart from unaccredited clinics.

What is AABB Accreditation?

AABB (Association for Advancement of Blood and Biotechnologies) is the internationally recognized leader in setting quality and safety standards for cellular therapies. AABB is a deemed status organization by the FDA and AABB accreditation represents the gold standard in the field — the most rigorous, comprehensive certification available for facilities that process and administer cellular products.

What Does AABB Accreditation Mean for You?

When you choose an AABB-accredited center like Auragens, you can trust that we meet the highest international standards in:

- **Cell Processing Excellence:** Our laboratory protocols follow strict, validated procedures ensuring every cell product meets pharmaceutical-grade quality specifications.
- **Donor Screening & Safety:** Comprehensive screening protocols ensure all donor tissue is

thoroughly tested and meets stringent safety criteria.

- **Laboratory Practices:** Our facilities, equipment, and processes undergo regular inspection and must meet exacting standards for sterility, environmental control, and quality assurance.
- **Patient Safety:** From cell arrival through administration and follow-up, every step is designed and monitored to protect your safety.
- **Staff Expertise:** Our team maintains specialized training and competency requirements specific to cellular therapy.
- **Traceability & Documentation:** Complete tracking and documentation of every cell product ensures full accountability.
- **Continuous Quality Improvement:** Regular audits, proficiency testing, and quality reviews.

Why This Matters

The field of stem cell therapy is rapidly growing, but not all clinics meet the same standards. Many facilities operate without independent accreditation or oversight. AABB accreditation means an external, U.S., FDA, expert organization has thoroughly inspected and validated our practices — giving you independent verification, proven quality systems, and genuine peace of mind. When you choose Auragens, you're choosing a center that has been independently verified to meet the most stringent international standards for cellular therapy quality and safety.

AABB accreditation is not easy to achieve or maintain — it requires significant investment in infrastructure, training, quality systems, and ongoing compliance. We've made this commitment because you deserve nothing less than the highest standard of care.

3. How hUC-MSCs May Help in Autism Spectrum Disorder

While the exact causes of ASD are still being studied, researchers have identified several biological processes that appear to be disrupted in many individuals with autism. hUC-MSCs are being investigated because they may help address some of these underlying issues. It is important to understand that this therapy is **investigational**—it is still being studied, and results vary from person to person. However, early research has identified several ways in which hUC-MSCs may support healthier brain and immune function in ASD.

3.1 Immunomodulation

Many children with ASD show signs of immune system dysregulation—meaning their immune system is overactive or not functioning properly. This can lead to chronic inflammation and may contribute to some of the behavioral and developmental challenges seen in autism.

hUC-MSCs have powerful **immunomodulatory** properties, meaning they can help "calm down" an overactive immune system. They do this by interacting with immune cells (such

as T cells, B cells, and macrophages) and releasing molecules that shift the immune response from a pro-inflammatory state to a more balanced, anti-inflammatory state [7].

In the context of ASD, this immunomodulation may help reduce systemic inflammation and create a healthier environment for brain development and function.

3.2 Neuroinflammation Reduction

Neuroinflammation refers to inflammation that occurs in the brain and nervous system. Studies have found that many individuals with ASD have elevated levels of inflammatory molecules in the brain, which may interfere with normal neural communication and contribute to symptoms.

hUC-MSCs are thought to reduce neuroinflammation by releasing anti-inflammatory factors that can cross into the brain and interact with brain cells. In one clinical study, children with ASD who received hUC-MSC therapy showed reductions in certain inflammatory markers in the blood, including macrophage-derived chemokine (MDC) and thymus- and activation-regulated chemokine (TARC), which are associated with immune activation. These reductions were observed in children who also showed improvements in autism symptoms, suggesting a possible link between reduced inflammation and clinical benefit [1].

3.3 Microglial Modulation

Microglia are the brain's immune cells—they act as the "cleanup crew" and help protect the brain from injury and infection. However, in ASD, microglia can become overactivated, releasing inflammatory molecules that may damage neurons and disrupt brain function.

Preclinical research (studies in animal models) has shown that hUC-MSCs can help modulate microglial activity, reducing their overactivation and shifting them toward a more supportive, anti-inflammatory role. In one study using a rat model of autism, intravenous hUC-MSCs reduced microglial activation in a brain region involved in social behavior and improved social interaction [8]. More recently, researchers found that extracellular vesicles (tiny packages of molecules) released by MSCs can reprogram microglial metabolism and reduce neuroinflammation through specific molecular pathways [10].

While these findings are promising, it is important to note that they come from animal studies, and more research is needed to confirm these effects in humans with ASD.

3.4 Paracrine and Exosome Signaling

One of the most exciting discoveries about MSCs is that they work primarily through **paracrine signaling**—meaning they release a variety of helpful molecules into their surroundings, rather than replacing damaged cells directly. These molecules include growth factors, cytokines (immune signaling proteins), and **exosomes** (tiny vesicles that carry proteins, RNA, and other cargo between cells).

Exosomes released by hUC-MSCs can travel through the bloodstream and even cross into the brain, where they deliver anti-inflammatory and neuroprotective signals to brain cells. Preclinical studies have shown that MSC-derived exosomes can reduce microglial activation, lower inflammatory cytokine levels, and improve social behaviors in animal models of autism [10]. These exosomes appear to work by interacting with specific receptors on immune and brain cells, helping to restore balance and support healthier neural function [9].

While this research is still in early stages and has not yet been rigorously tested in humans with ASD, it provides a strong scientific rationale for why hUC-MSCs may be beneficial in autism.

3.5 Oxidative Stress

Oxidative stress occurs when there is an imbalance between harmful molecules called free radicals and the body's ability to neutralize them with antioxidants. Some research suggests that oxidative stress may play a role in ASD, potentially contributing to cellular damage in the brain.

While preclinical studies have suggested that MSCs may help reduce oxidative stress through their secreted factors and metabolic effects [10], **there is currently insufficient evidence from human studies** to confirm that hUC-MSC therapy reduces oxidative stress in individuals with ASD. This remains an area of active investigation, and more research is needed to understand whether this mechanism contributes to any clinical benefits observed in patients.

3.6 Trophic Support

"Trophic" means "nourishing" or "supporting growth." hUC-MSCs release a variety of growth factors and supportive molecules—such as brain-derived neurotrophic factor (BDNF), vascular endothelial growth factor (VEGF), and nerve growth factor (NGF)—that may help support the health and function of neurons and other brain cells.

In the context of ASD, this trophic support may help promote healthier neural connections, support brain plasticity (the brain's ability to adapt and change), and create a more favorable environment for learning and development. While direct evidence of trophic effects in human ASD studies is limited, the known biology of MSCs and their secreted factors provides a plausible mechanism by which they may support brain health [7], [9].

4. Clinical Evidence and Research Findings

The clinical research on hUC-MSC therapy for ASD is still in its early stages, but several small studies and case reports have been published that provide important insights into

safety and potential benefits. It is important to understand that these studies are **preliminary**—they involve small numbers of participants, are mostly open-label (meaning participants and doctors knew they were receiving the treatment), and do not include placebo control groups. Larger, randomized, controlled trials are needed to confirm these findings.

Key Clinical Studies

Riordan et al. (2019) conducted a study in which 20 children with ASD received intravenous infusions of allogeneic (donor-derived) umbilical cord MSCs. Each child received 36 million cells every 12 weeks for a total of four infusions. The researchers measured autism symptoms using standardized rating scales, including the Childhood Autism Rating Scale (CARS) and the Autism Treatment Evaluation Checklist (ATEC).

The results showed that eight of the 20 children (40%) experienced meaningful improvements in their scores, with some moving from a higher symptom category to a lower one. The greatest improvements were observed three months after the final infusion. Importantly, the researchers also measured inflammatory markers in the blood and found that five of the eight children who improved showed reductions in MDC and TARC, two chemokines associated with immune activation. This suggests that the clinical improvements may have been related to reduced inflammation [1].

Sun et al. (2020) published a phase I safety and feasibility study in which 12 children with ASD received one to three intravenous infusions of human cord tissue MSCs (2 million cells per kilogram of body weight per infusion). The study was designed primarily to assess safety, but the researchers also measured changes in autism symptoms.

Six of the 12 children (50%) showed improvement on at least two ASD-specific outcome measures at follow-up. The treatment was generally well tolerated, though some children experienced mild infusion reactions or procedure-related agitation. Notably, five children developed new antibodies against donor tissue (anti-HLA antibodies), which were detected in blood tests but did not cause any immediate clinical problems. The long-term significance of these antibodies is not yet known [2].

Lv et al. (2013) conducted a non-randomized trial comparing two treatment groups to a control group receiving rehabilitation therapy alone. One group received cord blood mononuclear cells (CBMNCs), another received a combination of CBMNCs and umbilical cord MSCs, and the control group received standard care. Both treatment groups showed improvements in autism symptoms (measured by CARS, Aberrant Behavior Checklist, and Clinical Global Impression scales) compared to the control group at 24 weeks, with the combination group showing the largest effects [3].

Case Reports and Small Case Series have also been published describing individual children who received hUC-MSC or Wharton's Jelly MSC therapy and experienced improvements in language, motor skills, social interaction, or seizure control [4], [5], [6]. While these reports are encouraging, they are not controlled studies and cannot prove that the therapy caused the improvements.

What Does This Evidence Mean?

The current clinical evidence suggests that hUC-MSC therapy **may help** some children with ASD, particularly in areas such as social communication, behavior, and overall symptom severity. However, it is important to emphasize several key points:

- **Small sample sizes**: The studies published so far involve small numbers of participants (typically 12–37 children), which limits the ability to draw firm conclusions.
- **Open-label design**: Most studies did not include a placebo control group, so it is difficult to rule out placebo effects or natural improvements over time.
- **Variability in response**: Not all children improved, and the degree of improvement varied widely. Some children showed significant gains, while others showed little or no change.
- **Need for more research**: Larger, randomized, placebo-controlled trials with longer follow-up periods are needed to confirm these findings and to better understand which children are most likely to benefit.

A recent systematic review of the literature concluded that while the early evidence is "suggestive," it is "insufficiently robust" to establish efficacy, and called for more rigorous clinical trials with standardized outcome measures [8].

5. Safety Profile: What the Research Shows

Safety is a top priority for any medical treatment, especially when it involves children. The good news is that the clinical studies published to date have not identified any major safety concerns with hUC-MSC therapy for ASD. However, it is important to understand the limitations of the current safety data and to be aware of the potential risks.

What the Studies Have Found

Short-term tolerability has been generally acceptable across the published studies. Most children tolerated the IV infusions well, with only mild to moderate side effects that resolved on their own.

In the **Riordan et al. (2019)** study, the most commonly reported adverse events were mild and short-lived, such as temporary fatigue, mild headache, or brief changes in behavior. No serious adverse events were attributed to the cell therapy [1].

In the **Sun et al. (2020)** study, some children experienced mild infusion reactions (such as flushing or agitation during the infusion) or procedure-related anxiety. Importantly, five of the 12 children developed new **anti-HLA antibodies**—immune proteins that recognize the donor cells as foreign. These antibodies were detected in blood tests but did not cause any immediate clinical problems. However, the long-term significance of these antibodies is not yet known, and there is a theoretical concern that they could affect future transplants or immune responses [2].

In the **Lv et al. (2013)** study, no serious adverse effects were recorded during the monitoring period [3].

Case reports have generally described no serious side effects during the limited follow-up periods, though these small reports cannot detect rare or delayed adverse events [4], [5].

What We Don't Know

While the short-term safety profile appears favorable, there are important gaps in our knowledge:

- **Long-term safety**: Most studies have followed children for only a few months to a year after treatment. We do not yet know whether there are any long-term risks or delayed side effects.
- **Rare adverse events**: Because the studies are small, they may not detect rare but serious complications.
- **Immunogenicity**: The development of anti-HLA antibodies in some children raises questions about the potential for immune reactions, especially with repeated infusions or if the child needs a transplant in the future.
- **Optimal dosing and frequency**: It is not yet clear what dose, number of infusions, or treatment schedule is safest and most effective.

Auragens' Commitment to Safety

At Auragens, we take safety very seriously. Every hUC-MSC product we provide is:

- Sourced from carefully screened, healthy donors
- Processed in an AABB-accredited facility under strict quality controls
- Tested for sterility, viability, and potency
- Administered by trained medical professionals in a monitored clinical setting

We also maintain detailed records and follow-up protocols to monitor for any adverse events and to contribute to the growing body of safety data in this field.

6. What to Expect Next at Auragens

If you are considering hUC-MSC therapy for your loved one with ASD, it is important to know what the process will involve. At Auragens, we are committed to providing a safe, supportive, and transparent experience from your first consultation through follow-up care.

Initial Consultation

Your journey begins with a comprehensive consultation with one of our medical team members. During this visit, we will:

- Review your loved one's medical history, current symptoms, and treatment goals
- Discuss the potential benefits and limitations of hUC-MSC therapy
- Answer your questions about the science, safety, and what to expect
- Determine whether your loved one is a good candidate for this investigational therapy

Pre-Treatment Evaluation

If you decide to move forward, we will conduct a thorough pre-treatment evaluation, which may include:

- Physical examination
- Review of current medications and therapies
- Baseline assessments of autism symptoms using standardized rating scales
- Laboratory tests (blood work) to assess overall health and immune status

This evaluation helps us tailor the treatment plan to your loved one's specific needs and provides a baseline for measuring any changes after therapy.

7. Next Steps

If you are interested in learning more about hUC-MSC therapy for ASD or would like to schedule a consultation, we invite you to reach out to our team. We understand that this is a significant decision, and we are here to provide you with the information, support, and guidance you need.

How to Get Started

1. **Contact Auragens**: Call our patient services team or visit our website to request a consultation.
2. **Prepare Your Questions**: Write down any questions or concerns you have about the therapy, safety, costs, or logistics.
3. **Gather Medical Records**: If possible, bring copies of your loved one's medical records, recent evaluations, and current treatment plans to your consultation.

4. **Involve Your Healthcare Team**: We encourage you to discuss this option with your child's pediatrician, neurologist, or other healthcare providers. We are happy to communicate with your medical team to ensure coordinated care.

What to Consider

As you consider whether hUC-MSC therapy is right for your family, we encourage you to think about:

- **Your goals and expectations**: What are you hoping to achieve? Are your expectations realistic given the current state of the evidence?
- **Your loved one's overall health**: Is your loved one medically stable and able to tolerate an IV infusion?
- **Your support system**: Do you have the time, resources, and emotional support to commit to the treatment and follow-up process?
- **The investigational nature of the therapy**: Are you comfortable with the fact that this therapy is still being studied and that outcomes vary?

We are here to help you navigate these questions and to provide honest, evidence-based guidance every step of the way.

A Message of Hope

We know that living with autism—whether as an individual on the spectrum or as a family member—can be challenging. We also know that families are resilient, resourceful, and deeply committed to finding the best possible care for their loved ones.

While hUC-MSC therapy is not a cure for ASD, the early research suggests that it may offer meaningful benefits for some children by addressing underlying immune and inflammatory processes. We are encouraged by the clinical studies published to date, and we are committed to contributing to the growing body of knowledge in this field through rigorous, ethical, and compassionate care.

Every child with autism is unique, and every family's journey is different. Our hope is that by providing access to cutting-edge, evidence-based therapies—delivered with the highest standards of safety and quality—we can help open new doors and create new possibilities for the children and families we serve.

Thank you for considering Auragens as a partner in your journey. We are honored by your trust, and we look forward to the opportunity to support you and your loved one.

With hope and dedication,
The Auragens Team

The Auragens Patient Care Team

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