BEFORE THE
OFFICE OF ADMINISTRATIVE HEARINGS
STATE OF CALIFORNIA

In the Matter of:

CLAIMANT,

vs.

EASTERN LOS ANGELES REGIONAL CENTER,

Service Agency.

OAH No. 2021120572

DECISION

Cindy F. Forman, Administrative Law Judge (ALJ), Office of Administrative Hearings, State of California, heard this matter by videoconference on March 9, 2022.

Jacob Romero, Fair Hearing Coordinator, appeared on behalf of Eastern Los Angeles Regional Center (Service Agency or ELARC).

Claimant's mother (Mother) appeared on behalf of Claimant, who was not present. Claimant and Mother are not named for privacy reasons.

The ALJ heard testimony and received documentary evidence. The record was kept open until March 14, 2022, to allow for the submission of additional exhibits by Claimant and for ELARC to file a response. On March 9, 2022, Mother filed two

exhibits, an article titled "*Update on Umbilical Cord Blood Transplantation*" by Karen Ballen, published in 2017 in F1000 Faculty Reviews, and another article titled "*A History of Cord Blood Banking and Transplantation*" by Dr. Joanne Kurtzberg, published in May 2017 in Stem Cells Translational Medicine. The ALJ marked the articles as Exhibits N and O, respectively. ELARC filed its response on March 14, 2022, which the ALJ marked for identification as Exhibit 16. ELARC did not object to the admission of Exhibits N and O, and the ALJ admits the two exhibits into evidence as administrative hearsay. In addition, the ALJ admits Exhibit H, an article titled "*When the Alpha is the Omega: P-Values, 'Substantial Evidence,' and the 0.05 Standard at FDA,*" published in the Journal of Food Drug Law in 2017, as administrative hearsay.

The record was closed and the matter was submitted for decision on March 14, 2022.

ISSUE PRESENTED

Should ELARC fund an Expanded Access Protocol (EAP) umbilical cord blood (UCB) infusion for Claimant?

SUMMARY

Claimant presents with autism spectrum disorder (ASD). Mother seeks funding from ELARC to pay for a UCB infusion for Claimant to treat symptoms of his ASD. ELARC denied Claimant's parents' request because it considers the requested medical treatment to be experimental for the treatment of ASD symptoms. Although Mother presented various articles about the safety and efficacy of the requested treatment, she did not prove by a preponderance of the evidence that the medical community

considers a UCB infusion to be an accepted treatment for symptoms of ASD. ELARC's decision to deny Mother's request for funding to pay for a UCB infusion for Claimant is therefore upheld.

EVIDENCE RELIED UPON

Documents: Service Agency's exhibits 1 through 13, 15; claimant's exhibits A through O.

Testimony: Jacob Romero; Mother.

Jurisdictional Matters

1. Claimant is a four-year-old boy eligible for Lanterman Act supports and services based on his diagnosis of ASD.

2. At a time not made clear at hearing, Mother requested Service Agency to fund an EAP UCB infusion to improve Claimant's symptoms of ASD.

3. By notice of proposed action (NOPA) dated November 15, 2021, ELARC denied Mother's request, citing the Lanterman Act. The NOPA explained ELARC's clinical department had reviewed Mother's request and found the sought-after treatment "was still in the investigational phase." (Ex. 1, p. A1.) The NOPA cited Welfare and Institution Code (Code) sections 4434, subdivisions (a) and (d), 4646.4, subdivision (a), 4646.5, subdivisions (a)(1) and (a)(6), and 4648, subdivision (16), in support of its position. In particular, the NOPA made clear Code section 4648, subdivision (16), specifically prohibited ELARC from funding any experimental medical treatment and ELARC considered the UCB infusion an experimental treatment for symptoms of ASD. The NOPA also incorrectly noted there was a statutory exemption to Code section

4648, subdivision (16), although none exists. Once it recognized its mistake, ELARC sent a corrected NOPA, dated January 13, 2022, omitting the exemption. (Ex. 3.) Both the original and corrected NOPA fail to note that Code section 4648 had been amended as of July 16, 2021, and the amendment had renumbered subdivision (16) as subdivision (17). The amendment made no changes to the language of subdivision (16), and there is likewise no exemption for subdivision (17).

4. In response to the November 15 NOPA, Mother filed a Fair Hearing Request, which ELARC received on December 6, 2021. (Ex. 2.) On December 22, 2021, the parties held an informal conference to discuss Mother's request. After the informal conference, ELARC issued a letter affirming its denial of funding for the EAP UCB infusion.

Background

5. Claimant lives with his parents, brother, younger sister, and maternal grandmother. He needs constant care. He is nonverbal and unable to communicate with his parents. He presents with many challenging behaviors, including frequent temper tantrums ranging between several minutes to an hour long, biting, screaming, hitting, damaging property, elopement, engaging in self-injurious behavior, and difficulty sleeping. He requires supervision in all settings. He is not aware of any dangers around him. In addition to his diagnosis of ASD, claimant has been diagnosed with Global Developmental Delay and mitochondrial disorder.

6. Claimant requires help with all his self-care needs. He is not toilet-trained. He cannot hold utensils or feed himself. He needs assistance with dressing, teeth brushing, showering, and all hygiene tasks.

7. Claimant lacks socialization skills. He does not interact with other children. He keeps to himself most of the time when he is in a social setting.

8. Claimant has several gastrointestinal issues, has a B12 deficiency, and presents with severe constipation. He has been diagnosed with Pica. He also has several food intolerances, which require a special diet.

9. Claimant attends public elementary school from 9 a.m. to noon with a one-to-one full-time aide. He receives speech, occupational, and physical therapy at school.

10. Claimant's family transitioned to the Self Determination Program (SDP) on January 1, 2022. The family's total SDP budget is $32,085 and is divided between respite and financial management services.

11. Claimant's medical and dental needs are funded by private insurance and Medi-Cal. Private insurance and Medi-Cal also fund Claimant's Applied Behavior Analysis (ABA) therapy. Claimant's family receives support through In-Home Supportive Services.

Mother's Request for EAP UCB Infusion

12. Mother seeks ELARC funding to pay for an EAP UCB infusion provided by Duke University Medical Center (Duke) in North Carolina. According to Mother, the infusion is safe and effective and therefore should not be considered experimental. Mother contends no other therapy thus far has worked to ameliorate symptoms of Claimant's ASD, and she believes a UCB infusion, using Claimant's sibling's UCB, would help Claimant.

13. Mother provided ELARC with a Duke brochure about the UCB program and several articles to establish the safety and efficacy of UCB infusion for children presenting with ASD. According to the Duke brochure, the UCB infusion program is part of Duke's research efforts in developing new treatments to help children with brain injuries and certain neurologic conditions using UCB cells. (Ex. 8.) The Duke brochure explains its research has shown infusion of a child's own UCB (autologous infusion), or that of a full or partially matched sibling (allogeneic infusion), is safe. Duke is currently running clinical trials to determine if UCB and related products are effective in improving the symptoms of children with neurologic conditions, including ASD.

14. The Duke brochure further explains the United States Food and Drug Administration (FDA) has authorized Duke to offer UCB therapy to children with neurologic conditions, including ASD, by using their own UCB or UCB from a sibling, regardless of whether they qualify for a targeted clinical trial. The FDA authorization is based on the Expanded Access Program, which provides a means for patients with serious or life-threatening conditions to access investigational drugs outside of clinical trials. To participate, the patient must have a condition for which there is no comparable or satisfactory alternative therapy and where potential patient benefit justifies the potential risk. According to Mother, Claimant is eligible to take part in the EAP offered by Duke because of his ASD.

15. Duke charges $15,000 for each UCB infusion. It is not filing insurance claims for UCB services provided under the EAP. In addition to the infusion fee, parents are responsible for travel and lodging costs and the costs of shipping and storing the UCB. There are no discounts or payment plans associated with the program. Any medical complication caused by participation in the EAP requiring additional medical

care or medical services is the responsibility of the child's family and insurance provider.

16. Duke does not guarantee that a UCB infusion will improve a child's condition. The brochure states that a "potential benefit" of taking part in the protocol is the "possibility" that the UCB cells "may improve" a child's condition. (Ex. 8, p. A51.)

17. The articles offered by Mother establish UCB infusion to be safe. In an article entitled "*Update of Umbilical Cord Blood Transplantation,"* the author reports over 40,000 UCB transplantations have safely been performed to treat both children and adults with leukemia, lymphoma, and certain genetic disorders. (Ex. N.) According to the Duke brochure, Duke has performed more than 1,000 UCB infusions for children with brain injuries and related neurologic conditions. Of those, approximately 1.5 percent experience an allergic reaction during the infusion. These reactions are typically resolved by stopping the infusion and giving additional medications. In some cases, the UCB infusion can be resumed and completed, and in others, it cannot be completed. There are also theoretical possibilities of bacterial contamination and graft versus host disease in infusion recipients; however, the Duke brochure indicates the program has not seen these complications in any child treated. (Ex. 8, p. A51.)

18. In 2020, medical researchers analyzed the effectiveness of the Duke program for children with ASD and reported their results in a peer-reviewed article titled, "*A Phase II Randomized Clinical Trial of the Safety and Efficacy of Intravenous Umbilical Cord Blood Infusion for Treatment of Children with Autism Spectrum Disorder*," appearing in The Journal of Pediatrics in 2020 (Pediatrics Journal article). (Ex. 9.) Their analysis was based on a randomized, placebo-controlled, double-blind study including 180 children with ASD, aged two through seven, who received a single intravenous autologous (56 children) or allogeneic (63 children) UCB infusion versus a

placebo (61 children) and who were then evaluated at six months post-infusion. The results of the study showed UCB infusion was safe and well-tolerated. Analysis of the entire sample showed no evidence that UCB was associated with improvements in social communication, autism symptoms, or vocabulary. The researchers, however, found that a subgroup of the 180 children without intellectual disability showed significant improvements in communication skills and exploratory measures, including attention to toys and sustained attention, and increased alpha and beta EEG power, a measure of brain function. The researchers found no such improvements in those children who also had an intellectual disability. The researchers also noted that the study results may have been compromised because of the larger-than-anticipated number of participants with intellectual disability. (*Id.*, p. A60.) Based on their findings, the researchers concluded more research is called for to determine whether UCB infusion is an effective treatment for some children with ASD. According to the article, "The results of the present study do not currently support the use of [UCB] as a treatment for autism outside a formal or expanded access [investigational new drug]-sponsored clinical trial. Future research is warranted to determine whether [UCB] is an effective treatment for autism." (*Ibid.*)

19. Mother also offered letters by Frances Verter, the founder and director of the Parent's Guide to Cord Blood Foundation (Exhibit A), Meghna Desai, M.D., a board-certified hematologist and oncologist familiar with the Duke study (Exhibit B), and several parents who observed positive results after their autistic children took part in the Duke UCB clinical trials, in further support of the safety and effectiveness of UCB infusions for children with ASD. Mother offered no evidence showing any of these individuals know Claimant or have examined Claimant.

20. According to Ms. Verter, as of June 2021, the EAP at Duke had provided UCB infusions to 464 children, of which 278 had an established ASD diagnosis. Ms. Verter asserts the Duke UCB infusion program has shown to be effective for a subset of children with autism. She further writes that the treatment has "the potential to reduce [Claimant's] core autism symptoms, thereby improving his quality of life." (Ex. A, p. B3.)

21. Dr. Desai confirmed Ms. Verter's conclusion of the safety of UCB infusions. (Ex. B, p. B7.) In endorsing a UCB infusion for Claimant, Dr. Desai writes that Claimant "may stand to benefit from the Expanded access protocol at Duke University. As with any drug, we do not know yet which patient will benefit and why certain patients benefit over others but given a chance to improve their lives, all patients should be given an opportunity." (*Ibid.*)

22. Mother testified she and her husband have sacrificed their quality of life for Claimant. They have exhausted their savings and want to give Claimant the "best shot" in dealing with his disability. According to Mother, her family insurance denied Claimant's request to fund Claimant's participation in the UCB protocol at Duke because it considered the treatment to be experimental. Medi-Cal has also refused payment because the treatment is provided outside of California. She believes ELARC should fund the UCB therapy because it constitutes "specialized medical care" to alleviate Claimant's ASD, and ELARC is the payor of last resort.

23. Mother maintained the UCB treatment for ASD is not experimental. She asserted the results showing improvements for a subset of children presenting with ASD constituted substantial evidence that the treatment worked based on statistical analysis. According to Mother, because many of the study findings reflected a p-value of less than .05, the findings met the FDA threshold of efficacy. (See Ex. H.) She

acknowledged there was no guarantee the treatment would work for Claimant but contended that there is no guarantee for any treatment.

Testimony by Jacob Romero

24. Mr. Romero testified on behalf of ELARC. Mr. Romero asserted authorization of funding for the EAP UCB infusion would violate ELARC's Purchase of Service Guidelines for Health Services (Guidelines). (Ex. 5.) According to those Guidelines, ELARC will consider the purchase of specialized health services (i.e., services, supports, and adaptations of generic services directed at the alleviation of a developmental disability) under certain exceptional circumstances as needed for assessment or treatment. As a prerequisite of doing so, the regional center consumer must show no other source of payment is available. However, Mr. Romero explained that even if exceptional circumstances exist and insurance will not pay for the treatment, the Guidelines, consistent with Code section 4648, subdivision (a)(17), prohibit the purchase of experimental treatments. (*Id.,* p. A24.)

25. Mr. Romero consulted with Dr. Dolores Figueroa of ELARC's clinical team to evaluate whether the UCB infusion is an experimental treatment for ASD. As part of her evaluation, Dr. Figueroa reviewed Claimant's psychological evaluation, ABA treatment plan, the Duke brochure, the Pediatrics Journal article, and Mother's statement. Citing the Duke brochure and the Pediatrics Journal article, Dr. Figueroa determined UCB infusion therapy for ASD is an "investigational treatment." On that basis, Dr. Figueroa could not recommend ELARC funding the treatment. (Ex. 12.)

LEGAL CONCLUSIONS

1. The Lanterman Act governs this case. (Code, § 4500, et seq.) An administrative "fair hearing" to determine the respective rights and obligations of the consumer and the regional center is available under the Lanterman Act. (§§ 4700-4716.) Claimant requested a fair hearing to appeal ELARC's denial of funding for an EAP UCB infusion. Jurisdiction in this case was thus established. (Factual Findings 1-4.)

2. Because Claimant seeks benefits or services, he bears the burden of proving he is entitled to the services requested. (See, e.g., *Hughes v. Board of Architectural Examiners* (1998) 17 Cal.4th 763, 789, fn. 9; *Lindsay v. San Diego Retirement Bd.* (1964) 231 Cal.App.2d 156, 161.) Claimant must prove his case by a preponderance of the evidence. (Evid. Code, § 115.)

3. The Lanterman Act acknowledges the state's responsibility to provide services and supports for developmentally disabled individuals and their families. (§ 4501.) The state agency charged with implementing the Lanterman Act, the Department of Developmental Services (DDS), is authorized to contract with regional centers to provide developmentally disabled individuals with access to the services and supports best suited to them throughout their lifetime. (§ 4520.)

4. The "services and supports" provided to a consumer include specialized services and supports directed toward the alleviation of a developmental disability, or the social, personal, physical, or economic habilitation or rehabilitation of an individual with a developmental disability, or the achievement and maintenance of independent, productive, and normal lives. (Code, § 4512, subd. (b).) A regional center is required to secure the services and supports that meet the needs of the consumer, as determined in the consumer's individual program plan (IPP). (§ 4646, subd. (a)(1).) The

determination of which services and supports are necessary for each consumer must be made through the IPP process. (Code, § 4512, subd. (b).) The determination must be made based on the needs and preferences of the consumer or, when appropriate, the consumer's family, and include consideration of a range of service options proposed by individual program plan participants, the effectiveness of each option in meeting the goals stated in the IPP, and the cost-effectiveness of each option. (Code, § 4512, subd. (b).)

5. When purchasing services and supports for a consumer, a regional center must ensure, among other things, "[c]onformance with the regional center's purchase of service policies, as approved by [DDS] pursuant to subdivision (d) of Section 4434," and "[u]tilization of generic services and supports when appropriate." (Code, § 4646.4, subd. (a)(1) and (2).) Regional center funds "shall not be used to supplant the budget of any agency that has a legal responsibility to serve all members of the general public and is receiving public funds for providing those services." (Code, § 4648, subd. (a)(8).) If a service specified in a client's IPP is not available through a generic agency, the regional center may be required to fund the service if the service is necessary for the client to meet the goals set forth in the IPP. (Code, § 4648, subd. (a)(1); see also, Code, § 4659, subds. (a) and (b).)

6. Effective July 1, 2009, regional centers are prohibited from purchasing "experimental treatments, therapeutic services, or devices that have not been clinically determined or scientifically proven to be effective or safe or for which risks and complications are unknown." (Code, § 4648, subd. 17.) California law specifies that "[e]xperimental treatments or therapeutic services include experimental medical or nutritional therapy when the use of the product for that purpose is not a general

physician practice." (*Ibid.*) ELARC's Purchase of Service Guidelines incorporate this prohibition against funding experimental treatments. (Factual Finding 24.)

7. "Self-determination" means a voluntary delivery system consisting of a defined and comprehensive mix of services and supports, selected and directed by a participant through person-centered planning, to meet the objectives in their IPP. Self-determination services and supports are designed to assist the participant to achieve personally defined outcomes in community settings that promote inclusion. The SDP may only fund services and supports that the federal Centers for Medicare and Medicaid Services determine are eligible for federal financial participation. (Code, § 4685.8, subd. (c)(6).)

8. On June 7, 2018, DDS published guidance and definitions for the SDP regarding the consumer's purchase of certain goods and services (SDP Guidance). (https://www.dds.ca.gov/wpcontent/uploads/2019/05/SDP_serviceDefinitions.pdf.) Those goods and services, referred to as Participant-Directed Goods and Services, consist of "services, equipment or supplies not otherwise provided through the SDP Waiver or through the Medicaid State plan that address an identified need in the IPP (including accommodating, improving and maintaining the participant's opportunities for full membership in the community)." (Service Definitions, p. 18-19.) The purchase of those services and goods must meet the following requirements: "the item or service would decrease the need for other Medicaid services; promote interdependence, and inclusion in the community; and increase the person's safety in the home environment; and the participant does not have the personal funds to purchase the item or service and the item or service is not available through another funding source. The participant-directed goods and services must be documented in the participant's [IPP] and purchased from the participant's Individual Budget."

However, consistent with Code section 4648, subdivision 17, the SDP Guidance does not permit the purchase of experimental medical treatments, stating "Experimental or prohibited treatments are excluded." (*Id.*, p. 19.)

9. The Lanterman Act's prohibition of funding experimental treatments applies here. (Legal Conclusions 6–8.) Claimant has failed to show by a preponderance of evidence UCB infusion therapy is a generally effective medical practice for treating symptoms of children with ASD. Claimant has also failed to show that the therapy is not experimental. While the scientific community and clinical trials have established the safety of UCB infusions, the effectiveness of the therapy for ASD has yet to be determined. The therapy remains the subject of continued clinical studies conducted by Duke, the FDA has not approved the therapy for the treatment of ASD, and the findings from Duke's program thus far, as described in the Pediatrics Journal article, do not currently support the use of UCB therapy as a treatment for ASD outside of a clinical trial. (Factual Findings 13–19.)

10. Although there might be substantial evidence for certain clinical trial findings from Duke's program, i.e., the positive effect on UCB therapy on those participants with ASD who were not intellectually disabled, the impact of those findings is not clear. The number of clinical trial participants was small (180 children), and neither Duke nor the authors of the Pediatrics Journal article conclude that such limited evidence, particularly given the even smaller number of participants without intellectual disability, is sufficient to endorse the treatment without further study. (Factual Findings 13, 16, 18.) The possibility Claimant might benefit from a UCB infusion, as suggested by Ms. Verter and Dr. Desai, does not make the treatment less experimental. Anecdotal evidence of positive results from the UCB therapy is likewise insufficient to demonstrate the effectiveness of UCB transfusion therapy for ASD.

(Factual Findings 19–21.) Thus, there is currently insufficient empirical evidence to establish UCB infusion therapy meets the requirements of Code section 4648, subdivision (a)(17), and the SDP Guidance. (Legal Conclusions 6–8.)

11. Mother's testimony was credible, informed, and sincere. She wants the best treatment for her son, and she believes that a UCB infusion might be effective in improving the quality of his life. However, for the reasons discussed above, the Lanterman Act prohibits ELARC from funding this treatment. Because of that prohibition, it is not necessary to address the Service Agency's contentions regarding the absence of documentation showing denial of coverage for the infusion by the family's insurance and Medi-Cal and the extraordinary showing required for DDS to authorize funding for out-of-state services.

ORDER

Claimant's appeal of Eastern Los Angeles Regional Center's determination not to fund an Expanded Access Protocol Umbilical Cord Blood Infusion is denied.

DATE:

CINDY F. FORMAN
Administrative Law Judge
Office of Administrative Hearings

NOTICE

This is the final administrative decision; both parties are bound by this decision. Either party may appeal this decision to a court of competent jurisdiction within 90 days.