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TO: Maureen Wade, AHCCCS

RE: Stem Cell Transplant Policy Revision

Cyrcahealth Review & Recommendation

Cyrcahealth's stem cell transplant reviewers, Dr. Frank Smith of Cincinnati Children's Medical Center and Dr. Randy Wada of University of Hawaii, have both reviewed the proposed changes to the AHCCCS allogeneic stem cell transplant policy and made the following comments and suggestions:

1. <u>In regards to unrelated allogeneic stem cell or bone marrow transplants:</u>

Dr. Wada points to a lot of data that shows unrelated donor transplant can be effective. There are even some data suggesting that in some instances, unrelated donor transplant can have outcomes as good as related donor transplant. He is not aware of any data, however, that demonstrates the effectiveness of unrelated donor transplant in the context of a phase III trial. He is concerned about using phase III trial data as the criterion and feels this may be more of a cost-versus-benefit issue. However, for AHCCCS this concern may be more pertinent because reported survival for unrelated allogeneic donor transplants has been so low.

Dr. Smith noted that on page eight, no one uses GM-CSF anymore. He also suggested updating the covered transplants list and to include a mini-transplant (reduced intensity allogeneic transplants) narrative.

<u>2. In regards to the outcomes data for unrelated donor transplants for Arizona Medicaid:</u>

Dr. Wada said that both the NMDP and COG have performed survival analyses for small numbers, and while they show a trend for a relationship between numbers of transplants performed annually and survival, it has been hard to identify a "bright line" that determines the number of transplants that a center should do in order to perform optimally. Also, as the number of allogeneic transplants that a center does per year decreases, the confidence intervals that define performance levels become very large. He has a sense that while FACT has set a minimum number of transplants for certification eligibility, FACT has been placing increasing emphasis on the quality management aspects of certification.

One reason for the poor outcomes for Arizona Medicaid patients could be that the transplant center(s) involved don't have enough experience doing unrelated donor transplants. It could also be that they are transplanting higher risk patients, for example, those with resistant disease, or in relapse, or in minority/multi-race patients whose level of matching may not be as good as for Caucasian patients, even if the match looks good on paper. There may be a higher likelihood of minor mismatches because of ethnic differences between donor and recipient, or the mechanisms for follow-up of these patients are sub-optimal, due to their referral patterns and the lack of ability for the center to provide close follow-up care. It sounds like the patients all made it through the acute post-transplant period but then subsequently succumbed.

Some of these variables are addressed in the model the NMDP uses to evaluate transplant centers including patient acuity, disease status, match grade, etc. The NMDP then somehow factors this into the analysis and generates a confidence interval for "average" performance for that transplant center. It might be useful to get these analyses for the centers involved here.

Depending on the mix of patients they've transplanted, an option might be to require better match grades, or to more stringently define diseases and disease statuses that are eligible for coverage. One possibility would be to negotiate rates with Arizona transplant centers to see if they could be brought more closely to those for related donor transplant. In the end, even if outcomes could be improved, for example, by referring all transplants to outside centers with better performance, or if the costs within Arizona could be lowered either by competitive contracting or mutual agreement, could the costs be borne? It seems that these decisions are more of public policy rather than medical management and from Dr. Wada's personal perspective, he finds it hard to weigh the value of individual lives – he finds it easier from a healthcare provider's standpoint to sidestep that dilemma and concentrate on working as efficiently and effectively as possible with whatever resources he can get.

Per Dr. Smith, he felt the stem cell transplant policy in principle was okay, but that the real issue was the crude mortality rates for AHCCCS unrelated donor transplants. Even given expected rates per the CIBTR or NMDP, survival outcomes for AHCCCS patients are profoundly less than expected. He recommended AHCCCS immediately suspend payment for any further unrelated donor transplants due to quality concerns and poor outcomes. He also recommended an external independent audit of the adult unrelated donor bone marrow transplant programs, to include reviewing clinical records for all AHCCCS patients who underwent unrelated allogeneic donor transplants for the time period under review.

He recommends focusing on quality outcomes versus the impact of charges, not cost (page twenty -- billed amount) and that AHCCCS should seek an outcomes-based analysis from a quality of care standpoint. He also wonders what is the current, as of this date, NMDP and FACT status of the Arizona programs, noting that FACT certification has to be re-applied for anytime there is a change in transplant physician personnel. In other words, FACT certification cannot be "grandfathered" if the doctors involved with the original application are no longer with the program. Dr. Smith's main concern remains why indigent patients (Medicaid/AHCCCS) are

dying at a higher rate than expected, based on comparable data from the NMDP.

Recommendation: Otherwise, except for above discussion, proposed policy changes as submitted for review should stand.

References and Sources cited in the report were reviewed: No references to add.

If AHCCCS has any questions or concerns, please feel free to contact me.

Ronda Wells MD, CMO

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