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October 1, 2003

**RE: Proposed changes to UNOS Policy 3.7.6 (Status of Patients Awaiting Lung Transplantation), Policy 3.7.9 (Time Waiting for Thoracic Organ Candidates), Policy 3.7.9.1 (Waiting Time Accrual for Lung Candidates with Idiopathic Pulmonary Fibrosis (IPF)), and Policy 3.7.11 (Allocation of Lungs)**

To the Organ Procurement and Transplant Network (OPTN):

These comments are submitted on behalf of the Alpha-1 Association, the patient advocacy and support organization representing the community of individuals affected by Alpha-1 Antitrypsin Deficiency (Alpha-1) the fourth leading cause of lung transplants. It is important to start by expressing our appreciation for the work already completed on this important project which affects the future of so many with Alpha-1. In addition, please understand that we agree that the current, time-on-list based allocation needs to be replaced and believe the critical goal for any allocation scheme is to find a fair and just balance between efficacy and urgency.

We have reviewed the proposed lung allocation system of OPTN on several occasions. First, we reviewed it in preparation for the OPTN/UNOS public forum in March 2003 for the lung transplant community (primarily thoracic surgeons and pulmonary specialists). At that meeting, a representative of the Alpha-1 community publicly expressed concern regarding the allocation system as then proposed. We have noted that the allocation system proposal has been revised, supposedly based on the comments presented at the March meeting.

We have read what is available on the OPTN website and have had several physicians review this material as well. Unfortunately, the information provided on the website, while providing the wording of the proposed changes, does not make it easy to calculate how these changes will affect an individual patient in any detail. Even our medical consultants have been at a loss when trying to perform an actual calculation of patient priority status for a given set of characteristics.

The allocation system proposes the following broad scheme: Lungs will be allocated based on a score derived from the expected number of days of life following transplant minus twice the number of expected days of life without a transplant.\* No consideration whatsoever will be given to time on the waiting list. However, we anticipate that the following problems will arise from the implementation of this system.

First, the proposed system is based on “rule of rescue”, only looking at first year mortality on waitlist and 1<sup>st</sup> year post-transplant. As lung transplantation has become more successful, it is apparent that there are differences in long-term mortality between patients that cannot be predicted by their first year mortality. A system that targets waitlist and one year post transplant

mortality only will likely result in a net number patient life years lost because organs are not used for maximal utility.\*\*

Second, and most important to the Alpha-1 community, is the method by which the predictions of mortality are being made. Presumably because of the inability of our current medical technology to accurately predict mortality in a given individual with severe lung disease, patients will be grouped into four large "clumps" based only on their diagnoses (ignoring the different methodology proposed for children under 12). Group A will include Alpha-1 patients. Group A also includes individuals with COPD due to smoking, lymphangioleiomyomatosis, bronchiectasis, and sarcoidosis without pulmonary hypertension. It will be presumed that all of the various patients in a particular group have exactly the same life expectancy with and without transplantation. Small changes will be made in these numbers based on the patient's entering O<sub>2</sub> requirements, body mass index, age, FVC, and pulmonary artery pressures. Each of the other three "clumps" of diagnoses (Groups B, C, and D) has their own baseline predicted mortalities and measurements that adjust these baseline predicted mortalities up and down.

Of great concern is the fact that the major factor in determining the score will be the diagnostic Group (A, B, C, or D) and, as far as we've been able to ascertain from previous discussions, Group A will be given, automatically, one of the worst scores, primarily because individuals within this group tend get on the waiting list early and survive well, thus contributing a large negative number to the equation described above. Whether weighting factors such as FVC, age, body mass index, O<sub>2</sub> requirement, or pulmonary artery pressure will allow an Alpha-1 patient to move ahead of any patient in one of the other Groups remains to be seen. **Thus, with the information currently available to us, and the current small number of donor lungs available for transplantation compared to the large number of individuals on transplant waiting lists, it is possible that under this system, no one with Alpha-1 will ever receive a lung transplant!**

It is also unclear from the proposal how a sudden worsening of someone on the waiting list will be handled, since the score calculation is based on the score calculated at the time they join the transplant list.

One way of revealing the basic flaw in the proposed lung allocation system is by comparing it with the allocation systems of other solid organs. If a liver or heart transplant candidate is judged to have a low priority for transplantation but is "listable" then they are placed on the waiting list with many ahead of them. However, if this patient has a change in the status of their underlying disease and death is more imminent, they are reclassified and moved higher on the list. No matter how severe the lung disease of an Alpha-1 patient, he or she can never become sick enough to be reclassified as a cystic fibrosis or pulmonary fibrosis patient! Severity cannot change diagnosis, and diagnosis is the primary determinant of placement on the waiting list.



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In summary, the proposed allocation system as presented on the OPTN website could prove extremely detrimental to those waiting for a transplant with a diagnosis of Alpha-1. In view of the fact that, according to UNOS data, lung transplantation, especially double lung transplantation, has seen the highest long-term success rate in Alpha-1 patients compared with any other patient group, this seems especially unjust.

We strongly suggest immediate consideration of three strategies to facilitate the development of a just and equitable system in which equal consideration is given to reducing mortality as well as increasing utility for lung transplant allocation.

First, place a hold on the implementation of the current proposal pending completion of a study to evaluate the effects such system would have on all potential lung transplant recipients.

Second, ensure that any proposed change to the lung allocation system be stated in language that the patient community can evaluate completely and thoroughly.

Third, sponsor an open public forum to include end user patients and patient advocacy groups to discuss fair and equitable lung allocation, balancing efficacy and urgency, on lungs donated by the public\*\*\*

Thank you for your consideration of these comments.

Respectfully submitted,

Board of Directors, Alpha-1 Association

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\* It has been helpful to us to perform some simple calculations based on our understanding of the current proposal. If a calculation can be made for a given individual that he or she is likely to survive one year post-transplant and is likely to die in half a year without a transplant, their score would be 0 [365 days - (2 x 182.5 days)]. If a calculation can be made that a given individual will survive one year post transplant and one year without a transplant, their score would be -365 [365 - (2 x 365)]. If a calculation reveals that a given individual would survive a half year post transplant but one year without a transplant, their score would be -547.5 [182.5 - (2 x 365)]. Based on this, the person with a score of 0 would be transplanted before the one with a score of -365, who would be transplanted before the one with a score of -547.5.

\*\* Valapour, Maryam, MD, Bioethics Examiner, University of Minnesota, Spring 2003, Volume 7, Issue 1.

\*\*\* Dr. Arthur Caplan, Chair, Department of Medical Ethics, School of Medicine, and Director, Center for Bioethics, University of Pennsylvania endorses the call for a public meeting. The public which donates lungs does so in the belief that it is saving lives regardless for the reason for lung failure. It is absolutely crucial that all segments of those involved in lung transplantation- medical groups, donors, donor families and potential recipients and their families – fully understand and support the rules and algorithms used to allocate these scarce life-saving resources. He calls upon HHS to create a forum in which the allocation of lungs for transplant and for research can be openly and fully discussed.



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COPIES:

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The Honorable Thomas A. Scully, Administrator, Centers for Medicare and Medicaid Services

Michelle Snyder, Director, HRSA, Office of Special Programs

James Burdick, Director, HRSA, Office of Special Programs, Division of Transplantation

The Honorable W. J. "Billy" Tauzin  
Chairman, Committee on Energy and Commerce

The Honorable Michael Bilirakis  
Chairman, Energy and Commerce Health Subcommittee

The Honorable William Thomas  
Chairman, Ways and Means

The Honorable Nancy Johnson  
Chair, Ways and Means Health Subcommittee



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Background:

Alpha-1 Antitrypsin Deficiency (Alpha-1) is one of the most prevalent, potentially lethal hereditary disorders. Discovered in 1963, Alpha-1 can cause life-threatening lung disease and/or liver disease. It is a leading genetic killer of adults in the United States. Some 1600 Alphas have received transplants in the past decade (50% lung and 50% liver) which is as many as CF. Alpha-1 is also and a leading cause of liver transplants in children. It is estimated that 20 million Americans are undetected carriers of the Alpha-1 gene and may be at risk for lung or liver disease; 100,000 individuals are actively lung or liver affected and fewer than ten percent (10,000) have been accurately diagnosed. Once receiving an accurate diagnosis individuals with Alpha-1 lung disease may receive weekly intravenous augmentation therapy made from the pooled plasma of normal donors. End stage treatment includes lung transplantation.

Alpha-1 Association:

The Alpha-1 Association is a member-based nonprofit organization founded to identify those affected by Alpha-1 Antitrypsin Deficiency (Alpha-1) and to improve the quality of their lives through support, education and advocacy. For more information call 1-800-521-3025 or visit [www.alpha1.org](http://www.alpha1.org).