

Administrative Procedure Act, all proposed changes should be published with analyses before the Secretary makes a decision.

UNOS asked if the OPTN contractor would be required to submit to the Secretary for approval allocation policies in effect on the effective date of the final rule, pursuant to the process described in the final rule. For policies that the OPTN wants to be enforceable, the answer is yes. With the exception of particular policies established in this rule, all policies that have not been approved by the Secretary as enforceable remain voluntary, as explained in the 1989 Federal Register Notice. OPTN members that disagree with those policies may appeal them to the Secretary.

During the public hearing, a great many comments were directed to the question of the appropriate level of Federal oversight. While virtually all commenters agreed that the Department should have some role, opinions as to what that role should be varied from passive monitoring to taking very direct charge. Many of the particular suggestions made reflected the legal constraints that apply to organ transplantation. Some of these commenters also misunderstood the role and obligations of the Federal government for requirements that are established by law, even if implemented in part through private parties rather than by Federal staff. If the OPTN were a purely voluntary organization that happened to be a Federal contractor and if approved OPTN rules had no binding effect on patients or hospitals, then the appropriate level of oversight might be relatively low and limited primarily to efficient execution of the contract. But under the current law, patients have, as a practical matter, no choice but to use the system governed by the OPTN. Moreover, hospitals can lose the right to participate in Medicare and Medicaid and OPOs can lose reimbursement under Medicare and Medicaid for noncompliance with OPTN rules and requirements.

Both the genesis and wording of the National Organ Transplant Act (NOTA), as amended, obligate the Secretary to utilize the transplantation community substantially in both developing and executing transplantation policy. Under the statutory framework established by the Congress, however, the Department has oversight obligations, arising from the NOTA, as well as other laws and executive orders. For example, the Secretary has an affirmative obligation to make sure that policies and actions of the OPTN do not violate the civil rights of candidates for organ transplants. In this regard, however, most commenters stated, and the Secretary agrees, that Departmental oversight should not micro-manage the development of purely medical criteria or routine day to day decision-making of attending medical professionals or the OPTN contractor.

The Department, in the preamble to the proposed rule (59 FR 46486), made clear its intention to provide the public with an opportunity to comment on organ allocation policies and proposed changes to them. While we believe that the comment process administered by the OPTN itself is invaluable in obtaining technical advice, it does not reach all of the affected public--including potential donors and interested persons who are not OPTN members and have no access to the OPTN--or otherwise provide the functions and protections accorded by the impartial review by the Secretary. These principles are carried forward in the final rule. To allow sufficient time for public comment on policies that the Secretary decides to publish, we have deleted from proposed § 121.7(b)(3) the 30-day time limitation and have substituted "within a reasonable time." See, § 121.4(c)(2). The Secretary recognizes the importance of these issues, and expects the Department to act expeditiously on them. To ensure stability of the system, organ allocation policies, once implemented, continue to be in force during pending appeals or revisions.

New § 121.4 provides for an ongoing process of review that attempts to marry several goals: relying on the expert OPTN process to the maximum extent feasible; providing for independent review by the Department with additional opportunity for public comment; providing for cases where changes in policies may need to be made more rapidly than either process or both together would allow; and allowing the Secretary to take such other actions as the Secretary deems appropriate. Key to the effective functioning of this process is the acceptance by the transplant community of OPTN policies that have not been (and may never be) formally approved as enforceable requirements, but that most institutions choose to accept. A body of voluntary standards that can be rapidly revised, particularly for purely technical changes, is a crucial function of the OPTN system and one that the Secretary strongly supports. The Secretary believes that this rule puts in place an approach that accommodates all of the above goals.

7. § 121.8 - Allocation of organs

The majority of written comments received on proposed § 121.7 were opinions both for and against elements of the existing individual organ allocation policies, rather than comments on the content of this section of the proposed rule.

Several people discussed either the desirability or undesirability of permitting variances to current policies for allocating organs. Other commenters suggested broadening the geographic areas for organ allocation, localizing the areas for organ allocation, or allocating organs on a nationwide basis. One commenter said that allocation should be nationwide, because the current system is unfair to veterans. Under the medical coverage provided by the Department of Veterans Affairs (VA), veterans who need organ transplants are required by the VA to be listed with a transplant program with which the

VA has contracted. Another commenter said that local allocation is an important incentive to organ procurement and that the relationship should be studied. Another commenter objected to disparities in waiting time among geographic areas.

The American Society of Transplant Physicians suggested a conference to determine the suitability of patients for transplant, the establishment of standardized criteria to determine when a patient should be placed on the waiting list, and to define standards for a patient to be retransplanted. The United Network for Organ Sharing (UNOS), the OPTN contractor, provided a list of factors to be considered by the OPTN Board of Directors in developing organ allocation policies. All of these issues are addressed in this preamble. The Secretary notes that since the publication of the NPRM, some of these suggestions have been adopted.

The Secretary originally received 62 letters commenting on organ allocation policies, of which 50 were about the lung allocation policy (many of those concerning lungs were form letters from patients at a single institution). These commenters, most of whom were individuals identifying themselves as organ transplant recipients, potential recipients, and friends or relatives of potential recipients, urged that geographic areas for lung allocation be broadened to permit more organs to be allocated to a particular medical program.

Comments on other organ allocation policies were also received from individuals affiliated with hospitals, from the American Society of Transplant Physicians, from the Cystic Fibrosis Foundation, from a law firm representing a hospital, and from a member of Congress on behalf of a constituent. Two comments were on the kidney allocation policy, one supporting local allocation and the other providing a copy of technical comments sent to the OPTN on revising the point system. One comment was on the heart allocation policy, suggesting that the geographic boundaries for allocation under the current policy

be made more flexible. Two comments were not specific with respect to a particular organ, but recommended that allocation be nationwide based on time on the waiting list.

The Secretary also received letters urging action on liver allocation with emphasis on wider sharing. These comments, and many others on related allocation issues, arising both in the original comment period and at the public hearing, are addressed below in our proposed performance goals.

When the proposed rule was issued in 1994, the Department posed several open-ended questions about allocation policy, with the expectation that public response would help us decide how best to handle allocation policy and the extent to which we would seek to establish such policy in this final rule or in policy-by-policy reviews. Both in the initial set of public comments and in the months surrounding the public hearing, the Department received a great deal of information about, and many criticisms of, current allocation policies. For example, we learned that current allocation policies, by allowing local geographic boundaries to override patient needs, do not follow an ethical opinion addressing this very issue, promulgated through the Code of Medical Ethics of the Council on Ethical and Judicial Affairs of the American Medical Association. Second, we received the early results of computer modeling sponsored independently by UNOS and the University of Pittsburgh Medical Center (UPMC). These modeling efforts provided quantitative estimates of a great many variables--lives saved both pre- and post-transplant, time on waiting list, graft survival rates, etc.--that had previously been difficult to address systematically when alternative allocation policies were compared. Third, the OPTN itself continued to study, debate, and consider major revisions to its policies. Building on this new information, a primary purpose of the December hearings was to obtain even more information and opinions on organ allocation policies, particularly those affecting livers. That purpose was achieved.

Based on these sources and much other information, the Department has determined that the original proposal in the NPRM was insufficient. The transplantation community is very divided, on allocation policy in general and specifically on liver allocation, and the existing policy development process is unlikely to bridge those divisions. Medical issues, ethical issues, and matters of trust and actual practice are substantially intertwined. Yet, the Department is unwilling, at this time, to issue a prescriptive allocation policy. We believe the OPTN must be primarily responsible for establishing medical criteria for patient listing and status categories, and for developing equitable allocation policies that reflect the Secretary's policies, as expressed in this regulation.

The Secretary decided, therefore, to approach the issue in terms of performance goals. The basic idea of a performance goal is to set a target, allow the operating entity (in this case the OPTN) to determine how best to meet that goal, and then measure performance against that goal. This model is widely used in business and in public programs. It is the model for this Department's Healthy People 2000 goals and other initiatives as well as the recently enacted Government Performance and Results Act. Quite apart from its other advantages, it promises to clarify and strengthen the Department's review and approval process for OPTN policies.

Based on the detailed and helpful dialogue at the hearing, and the clearly expressed preferences of commenters on both sides of specific issues, the Secretary has determined that three broad performance goals for organ allocation are needed. The topics of these goals are: (1) minimum listing criteria, (2) patient status, and (3) priority for patients with the highest medical urgency. The Secretary has also added a requirement, discussed below, for the OPTN to assess the cumulative effect of its policies, and develop new policies as appropriate, regarding socioeconomic equity. All of these goals are subject to sound medical judgment, both as to specific patients and as to overall standards, in order to

avoid organ wastage, reflect advances in technology, and otherwise operate an effective and efficient allocation system.

Listing (§121.8(a)(1)). Many commenters at the hearings pointed out that current allocation policies (which give substantial weight to overall waiting time without regard to status) encourage aggressive physicians to list patients for transplants as early as possible, in some cases years before they will need or want a transplant. Other physicians are more conservative, and some patients do not come to the attention of transplant professionals until later in the course of their underlying condition. As a result, persons with equal waiting times may have very different medical urgency. This means that overall waiting time as a “tie-breaker” is unfair, encourages “gaming” behaviors and distrust within the transplant community, and discourages sharing of organs across geographic areas (because a less needy patient in one local area may obtain preference over a more needy patient in another local area simply by virtue of aggressive early listing). We have determined, therefore, to require that the OPTN develop listing criteria that are based on objective medical criteria pertinent to each organ, and to update these criteria to reflect increasing medical knowledge. The OPTN already has efforts under way that go a long way toward achieving this objective, and the Secretary applauds those. As explained below, overall waiting time will also be replaced by waiting time in status as a “tie breaker.”

Patient Status (§121.8(a)(2)). Another set of themes emerging from the hearings is the recognition that current liver allocation criteria fail to differentiate adequately among different degrees of medical urgency and the desire for substantial improvements in the use of objective medical criteria for the classification of patients. In some cases, existing criteria are based on situational factors, such as whether a person is hospitalized, which are neither medical criteria nor necessarily good proxies for underlying medical condition or urgency. They can also encourage choices on the part of managing

physicians to make sure that their own patients are not disadvantaged relative to other persons. At the same time, we know that advances in transplantation medicine and the OPTN's extensive investment in patient information systems have made possible improvements in the classification of patients. The ever-improving knowledge base about the medical factors that correlate with transplant outcomes, combined with the use of computer technology and statistical analysis, allow sophisticated ranking of patients, without the need to group disparate patients into relatively few and crude categories. The Secretary has decided to endorse the requested reforms and require improved categorization of patients, based on objective medical criteria that distinguish among different levels of urgency in sufficient detail as to reduce discriminatory effects.

Priority for the Most Urgent and Geographic Equity (§121.8(a)(3)). By far the most controversial aspect of current allocation policies is that the "local first" feature creates inequities in access for organs among patients of equal medical urgency, making where they live or list a more important factor than objective measures of medical status in obtaining an organ. All patients are affected by these inequities, but the consequences fall most heavily on those whose medical need is greatest and who are most likely to die before receiving an organ. As shown in tables 3a and 3b below, there are vast differences in median waiting times for kidneys among different transplant programs and different organ procurement areas (table 3a addresses transplant hospitals and is adapted from OPTN data printed in the *Cleveland Plain Dealer* on February 5, 1997; table 3b addresses organ procurement areas and is adapted from OPTN data on waiting times that will shortly be published):

Table 3a
Shortest and Longest Waiting Times by Kidney Transplant Program
1994-1995

Shortest Hospital Waiting Times:	Median Waiting Times (Days)
Harris Methodist, Fort Worth, TX	54
Presbyterian-University, Pittsburgh, PA	79
Southwest Florida, Fort Myers, FL	114
Henrietta Egleston, Atlanta, GA	144
Oregon Health Sciences, Portland, OR	147
Longest Hospital Waiting Times:	
University of Pennsylvania, Philadelphia, PA	822
Northwestern Memorial, Chicago, IL	828
Lehigh Valley, Allentown, PA	838
William Beaumont, Royal Oak, MI	850
Milton Hershey, Hershey, PA	858

Source: Cleveland Plain Dealer, February 5, 1997, reporting UNOS data.

Table 3b
Shortest and Longest Kidney Transplant Waiting Times by Local
Allocation (OPO) Area
1993-1995 for blood type O

Shortest OPO Waiting Times:	Median Waiting Times (Days)
Oregon Health Sciences University Hospital	107
Lifelink of Southern Florida	143
Lifelink of Florida	161
Life Connection of Ohio	204
Longest OPO Waiting Times:	
Carolina Organ Procurement Agency	1,423
Regional OPA of Southern California	1,501
California Transplant Donor Network	1,513
New York Organ Donor Network	1,680

Source: UNOS data, soon to be published in report on waiting times. The OPO waiting times are longer than hospital waiting times mainly because type O patients wait longer than most other blood types.

Unfortunately these data, although the best available, do not isolate the differences in patient condition or in transplant centers listing practices that underlie some of the observed disparity. For example, as discussed previously, some doctors aggressively list patients very early in the course of their disease to give them more waiting time and raise their chance of obtaining an organ. Such a practice artificially inflates waiting times in some areas. However, the differences in waiting times by area far exceed the differences in medical status by area.

These differences exist throughout the United States. As shown in Table 4, each OPTN region has many local OPO allocation areas with relatively short and relatively long waiting times:

Table 4
Range of Kidney Transplant Waiting Times Among OPOs by OPTN Region
Median Waiting Time in Days, 1994 for blood type O

Median Waiting Times for Kidneys	Days (shortest-longest)
Region 1 (New England)	413-1,360
Region 2 (DC, DE, MD, NJ, PA, WV)	702-1,378
Region 3 (Southeast)	143-761
Region 4 (OK, TX)	386-655
Region 5 (California & Southwest)	374-1,513
Region 6 (Northwest)	107-1,061
Region 7 (Upper Midwest)	794-1,176
Region 8 (CO, IA, KS, MO, NE, WY)	287-754
Region 9 (NY)	228-1,680
Region 10 (IN, MI, OH)	204-1,422
Region 11 (KY, NC, SC, TN, VA)	231-1,423

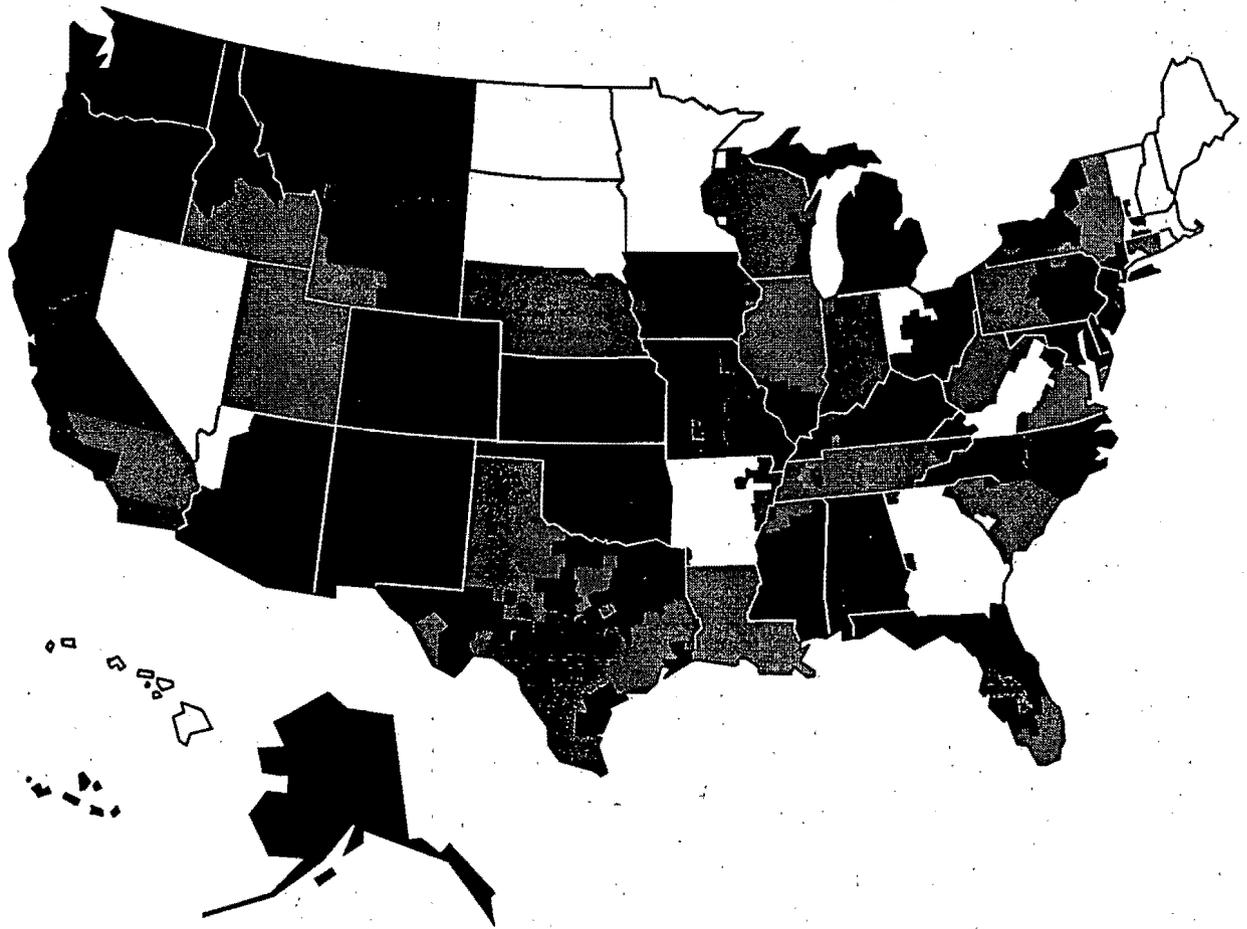
Source: UNOS data, soon to be published in report on waiting times

Similar waiting time differences exist for other organs. To some degree, these differences in waiting times result from the current absence of standardized listing criteria, as discussed above. Hence, these are imperfect measures of differentials. They also reflect, however, the fact that current patients who happen to list in areas with either higher incidence of end stage organ disease, or less ability to generate organ donors, are systematically disadvantaged by policies that do not permit the organs to go to the patients who need them the most. They also work to the disadvantage of prudent purchasers who wish to designate or contract with particularly high quality (or low cost) transplant hospitals to serve their patients. Under current allocation policies, neither individual patients nor concerned payers have the

freedom to select their preferred medical provider without, in many cases, increasing the chance that the patient will wait longer and die while waiting for an organ.

Individual patients are directly affected, regardless of medical need. Although the Department is mindful that anecdotes can be misleading, the following example illustrates the inherent effects of establishing unduly restrictive geographic barriers to equitable organ allocation. In a recent case reported in the press (*Sunday World Herald* of Omaha, Nebraska, May 25, 1997), a patient was forced to choose between listing with a "local" hospital 250 miles away but in an organ procurement area that covered his State and had access to relatively more organs, or with his strongly preferred and truly local hospital just 20 minutes across a river and in another State that had access to relatively fewer organs. Cases such as this are inherent in a system that established defined areas for the purposes of administering organ procurement, but whose boundaries also have been used to limit organ allocation. Reliance on boundaries that make sense for administrative convenience may lead to inequities in organ allocation criteria. For example, in a number of States one OPO is surrounded by another; and in Texas there is an OPO that is composed of four non-contiguous areas separated by other OPOs. Some OPOs are based on the service area of a single hospital; some follow the boundaries of a single State; and others serve four or more States. These and other vagaries of this system are shown in the following map. Because of the differences in OPO size, geography, and population, the Secretary has decided that OPO areas should not be the primary vehicle for organ allocation.

Organ Procurement Organization Service Areas, 1997



Payers are also directly affected. Their ability to select transplant hospitals for their patients is hampered if listing patients solely at those hospitals forces them to compete with local patients for the limited supply of local organs, even though this listing frees up organs in the areas in which the patient would otherwise be listed. Some large payers have tools at their disposal to ameliorate this problem, such as listing some patients at more than one center (multiple listing), listing some patients at centers with shorter waiting lists, or accelerating hospitalization to put patients in a preferred status. However, most payers do not use such techniques and only a minority of patients benefit from such "gaming."

Perhaps the greatest inequity that the current system of local priority creates is that it particularly disadvantages those who face imminent death through unusually rapid deterioration. The chance that an organ that will match one's physiology will be available in the local area within the next week is very small. Yet, the chance that an appropriate organ will be available somewhere in the country and that it can be transported without risking wastage is much higher.

The transplant community has differing opinions over the issue of broader sharing. According to some commenters, this is in part because some hospitals and their patients reap the benefits of a highly productive OPO and they are concerned that they may receive fewer organs under a national system. Many commenters have pointed out that local preference draws upon, and reinforces, close bonds among local organ procurement organizations and local hospitals and physicians. Almost all agree that there are logistical and practical reasons why organs cannot be shipped back and forth across the country in response to the daily needs of every individual patient.

As shown below in Table 5, there are great disparities among OPOs in the production of donor organs, and under the current system, the productivity of the local OPO directly impacts on the number of transplants done in the OPO service area.

Table 5

Donors Per Million Population 1995

<u>Donors Per Million Pop.</u>	<u>Percentage of OPOs</u>
<15.00	19.4
15.00 - 20.00	22.4
20.01 - 25.00	37.3
25.01>	20.9

Note: The range of OPO donors per million population is 6.4 to 31.6

Source: Calculation by the Division of Transplantation using UNOS Data

Major review agencies, including the Inspector General of this Department and the Congress' General Accounting Office, have reviewed allocation issues and issued reports concluding there are major inequities and that major reform is needed to make the allocation system a truly "national" system as intended by the Congress.

The American Medical Association has studied organ allocation through a panel of experts. In its *1996 Code of Medical Ethics* it states that: "Organs should be considered a national, rather than a local or regional, resource. Geographical priorities in the allocation of organs should be prohibited except when transportation of organs would threaten their suitability for transplantation." In reaching this conclusion, the AMA panel reviewed the evidence concerning several organ types, and a wide range of alternative formulations. Of particular importance was their finding that current organ allocation policies were, in

some cases, seeking to favor patients of lesser urgency but more likely to benefit, but that in actual practice these benefit differences were far too small to justify differential priority.

Taking all of these arguments into account, the Secretary has determined that a national performance goal is needed to encourage the OPTN to take advantage of advances in technology and survival rates, and to bring policies in line with the intent of the National Organ Transplant Act. That goal would reduce geographic inequities by requiring that persons with equal medical urgency (i.e., in the same status as defined under the second performance goal) have essentially equal waiting times regardless of where they list. This standard emphasizes, however, that the sickest categories of patients should receive as much benefit as feasible under this standard, in accordance with sound medical judgment. This is a significant departure from current policies, not only in making geography less important for allocation purposes, but also in its approach to waiting-time disparities. The relevant “tie-breaker” will no longer be total waiting time, perhaps years, but will become waiting time within a group of patients with equal medical urgency.

We are mindful that there are practicalities involved, including especially transportation. The problem is not occasional cross-continental shipping from one large city to another, which is relatively straightforward. Instead, however, there can be severe logistical problems with frequent shipping of organs (often preceded by a special team that travels to retrieve the organ and return with it), or with moving organs among relatively transportation-disadvantaged areas, even within the same State. The performance goals are designed to allow (and require) the OPTN to craft policies tailored to each organ transplant type that are workable, feasible, and avoid organ wastage.

Many commenters urged that the Secretary require national sharing of organs, without any role for geographic factors. Others urged regional sharing. We prefer the performance goal approach. Achieving

the goal will certainly require greater geographic sharing and will probably require national sharing for some organs for patients with specified medical conditions. Indeed, regional sharing is already a prominent feature of heart allocation, and national sharing a prominent feature of kidney allocation. However, we believe that any simple formulation would inhibit the ability of the OPTN to craft the most sensible policies that achieve practical as well as ethical results, and we wish to encourage change over time as medical science and medical criteria improve. Therefore, we are at this time using the performance goal approach for all organs (with an accelerated schedule for the initial revision of policies for liver allocation).

Implicit in the requirement that patients with equal medical urgency and waiting time in status have an equal chance of receiving an organ is reform of policies that encourage organs to be diverted from patients of blood type O, the "universal donor," in favor of patients of other blood types, if that would preclude equalization of waiting times in status. One of the inequities of present organ allocation policies is that patients of blood type O wait much longer for organs than other patients. For example, according to recently calculated data from the OPTN, the median waiting time for primary kidney transplants in 1994 was 824 days overall, but 1,007 days for patients of blood type O. For hearts, the median waiting time was 224 days overall, but 353 days for patients of blood type O in 1996. Blood type is not an indicator of medical urgency, although it is a key determinant in organ matching.

The Secretary appreciates that there are many factors that can contribute to achieving the geographic equity goal. For example, if the Department's organ donation initiative were to achieve a high rate of success, then fewer organs would need to be shared. Improved listing criteria and medical status categories will reduce measured inequities. Nonetheless, within foreseeable parameters, we see no

basis to expect that inequities can be eliminated for any major organ category without broader geographic organ sharing, on at least a broad regional basis for all patients with high levels of urgency.

We also require the OPTN to take into account key constraints on organ allocation. There are patients with urgent need for whom transplantation is futile. Organs cannot be used without an assessment of the immune system and other physical conditions of patients. Broad geographic sharing should not come at the expense of wasting organs through excessive transportation times. Efficient management of organ allocation will sometimes dictate less transportation when the highest ranking patient can wait a day or two for the next available organ. Sound medical judgment must be exercised before a final decision on whether to transplant a particular organ into a particular patient. Our goals allow for these factors to affect transplantation outcomes. For example, current OPTN policies take into account the special medical needs of children. The Secretary endorses this approach and expects that the OPTN will continue to take these needs into account as it develops new medical criteria and allocation policies.

Transition Protections (§ 121.8 (a)(5)) Finally, we have added a requirement that transition protections (sometimes termed “grandfather” rights) be considered whenever a change in policy disadvantages an identifiable set of patients already waiting on the national list of transplant candidates.

To implement these protections, the OPTN would determine whether a change disadvantaged some patients, and if so, consider developing a transition policy to eliminate that disadvantage. The transition policy would be submitted to the Department for review along with the new policy, together with estimates of the likely effects of each. Because a transition policy complicates organ allocation, and because the Secretary wants to preserve OPTN flexibility to develop and implement minor improvements with no consequential effect on existing patients’ priorities, the transition provision allows the OPTN

some flexibility as to whether, for how long, and for which patients the transition procedure would be developed. Of course, the OPTN would be free to devise particular approaches that would be most efficient and effective for a particular patient population. As with all other allocation policies, the Department would review each proposed transition procedure.

In addition, the Secretary has adopted a special transition provision for the first revision of the liver allocation policy. The OPTN is directed to develop a transition proposal for the Secretary's review which would, to the extent feasible, treat each individual on the national list and awaiting transplantation on the date of the publication of this regulation in the Federal Register no less favorably than he or she would have been treated had the revised policy not become effective. The transition procedures for this initial revision of the liver allocation policy may be limited in duration or applied only to individuals with greater than average medical urgency if this would significantly improve administration of the list or if such limitations would be applied only after accommodating a substantial preponderance of those disadvantages by the change in the policy. See § 121.8(a)(5)(ii).

Kidneys pose potential problems because, unlike other organs, a significant fraction of patients have already spent years on the national list and turnover is much lower. On the other hand, transition procedures may be particularly important for kidney patients for the same reason. We request comments on the transition procedure generally and specifically as to its suitability for kidney patients.

(a) Indicator Data (§ 121.8 (a)(4) and 121.8 (b)) In order to assess how well the OPTN's current or proposed allocation policies achieve the performance goals previously stated, the Secretary requires the OPTN to collect and report indicator data on outcomes, and to compare alternative policies against estimated or projected outcomes. It is primarily against these indicators that the Secretary will determine

whether the OPTN's proposed revisions to organ allocation policies will be approved. The Secretary expects the OPTN to develop appropriate indicators, but has specified several of central concern. These are: disparities in waiting times in status among transplant programs (especially disparities among the sickest categories of patient); life-years lost (both pre- and post-transplant); the number of patients who die while waiting for a transplant, and the number of patients mis-classified. Our requirements for performance indicators are presented in § 121.8(a)(4). See also, §121.8 (a)(3), discussed earlier, for the allocation policies themselves.

Over the past year, a great deal of the debate and analysis of alternative allocation policies has benefitted from the results of computer-based modeling of liver allocation. While current modeling has some limitations, it is nonetheless useful today and holds great promise of assisting the OPTN in devising, as well as assessing, policies. The Secretary expects the OPTN to develop and use such models for all organs and to present results to the Department.

(b) Deadlines for Initial Reviews (§121.8(c)) The Secretary expects the achievement of these goals to be an ongoing process as medical technology, experience, and our understanding of transplantation improve over time. Therefore, we have provided for periodic policy revisions. However, for all organs other than livers, the Secretary is requiring that the OPTN develop initial revised policies to meet the goals, and to submit these within one year from the effective date of this rule. For livers, the Secretary is requiring development of policies that will meet these goals, to be submitted by 60 days from the effective date of this rule.

Shortly after this deadline the Secretary will take action with respect to the OPTN liver allocation proposal, depending on the information available to us as to which option best meets the performance

goals set out in this rule. During consideration, the Secretary is committed to using a process allowing for effective comment and presentation of alternatives. In order to minimize the time needed to develop approved policies, the Secretary will follow carefully the OPTN's progress in developing the new liver allocation policies.

(c) Liver Allocation Policies The OPTN has wrestled with liver allocation issues for a decade. A brief summary of this history helps in understanding both the current OPTN policy and the Department's approach in this regulation. One of the two main purposes of the December hearing was to obtain additional information and views on liver allocation.

UNOS adopted a liver allocation policy in 1986, the first year of OPTN operations. The allocation policy featured a point system assigning relative weights for medical urgency, blood group compatibility and waiting time to patients within distinct distribution units. This initial system allocated organs first among all patients locally (with "local" waiting lists meaning the OPO procurement area, ranging from a single transplant hospital's list to the combined lists of all transplant hospitals in an entire State), then to patients in the OPTN region. At the time this policy was adopted, the country was divided into nine regions. Eventually, the number of regions was expanded to the current eleven to reduce differences in population size among the regions. Major differences still remain, however.

The liver allocation policy also included an informal emergency voluntary sharing practice known as "UNOS STAT" whereby a transplant hospital would notify the UNOS Organ Center (the 24-hour organ placement operation maintained by UNOS) that a patient was critically ill and expected to die within 24 hours without a transplant. The Organ Center, in turn, would immediately notify all OPOs and transplant programs of the urgent need. Should a liver become available, the OPO could bypass the usual

allocation process and the liver could be directed to the UNOS STAT patient's hospital. In effect, UNOS STAT was a system for sharing livers nationally, but only for the medically neediest patients. Between 1987 and 1990, it is estimated that 15 percent of the patients who received transplants were designated as UNOS STAT.

Objections were raised about the use of UNOS STAT, citing a lack of formal, uniform rules governing its use, and a concern that it was being used excessively or inappropriately. It was abolished by the OPTN in 1991. In addition to eliminating the UNOS STAT category, the liver allocation policy modified in 1991 expanded significantly the definition of the most urgent category by redefining it to mean death within seven days without a transplant (rather than 24 hours as in UNOS STAT). The rationale for the change was to provide greater opportunity within the formal allocation system for transplantation of chronically ill patients as well as those with acute fulminant liver failure.

Waiting time accrual under the liver allocation criteria was also modified to give greater priority to the most urgent patients. Status 1 (originally Status 4; in the discussion the sickest patients will always be referred to as Status 1, the current definition) patients were assigned the highest priority within the same distribution unit by only allowing waiting time accrued by a patient while listed as Status 1 to count for liver allocation. The Status 1 criteria specified until recently that such patients have a life expectancy of less than 7 days without a liver transplant. Patients who are listed as Status 1 automatically revert to Status 2 after 7 days unless they are relisted as Status 1 by an attending physician. Prior to this policy change, it was possible for a patient who had been waiting a long time in a lower status to accumulate enough waiting time points to give that patient enough total points to be ranked higher than a patient who was a Status 1. The definitions of Status 2, 3, and 4 patients were, until changed, as described below:

Status 2: Patients are continuously hospitalized in an acute care bed for at least 5 days, or are in the intensive care unit. Continuous hospitalization is required.

Status 3: Patients require continuous medical care but may be followed at home or near the transplant hospital.

Status 4: Patients at home, functioning normally.

However, because the system allocates organs first locally, then regionally or nationally only if no local patients are a good match for the organ, and because at any time it is likely that the relatively few (or no) local patients in Status 1 will match, many organs go to Status 2 and 3 patients despite their being ranked lower in medical priority. In the mid 1990s, about two thirds of liver transplants were received by patients waiting in the "local" area, about one fifth by patients in the region and outside of the "local" area, and about one eighth by patients outside the region. Therefore, the preference for "local" plays a significant role in determining a patient's likelihood of receiving an organ. Under the current system, there is a wide range among OPOs and the OPTN regions in the number of patients on the waiting list, the number of donor livers available, and the ratio of patients per donor. Consequently, patients in different locations have disproportionate probabilities of being offered a liver under this arrangement. Further, because fixed boundaries are used in local and regional distribution, some patients nearest the site of the donor who are otherwise highly ranked according to urgency or waiting time continue to wait while less sick patients in the "local" region are transplanted. As a result, some patients with higher medical urgency die waiting for a liver while other patients with less medical urgency receive a transplant.

Between 1990 and 1996, the number of liver transplant hospitals performing at least one liver transplant increased from 75 to 110, and the number of liver transplant programs performing 35 or more

liver transplants per year increased from 18 to 41. Liver transplants increased from 2,676 to 4,012. Thus, patients have more transplant hospitals from which to choose, but at the same time competition among liver transplant programs for available livers has increased. During 1996, there were 8,026 registrations for a liver transplant.

Some people criticize this policy because livers are allocated "local first" to whomever is highest ranked in the local area of procurement. Thus, less sick patients can be transplanted before sicker patients in other local allocation areas. They believe that the sickest patients should always be transplanted first regardless of their location, because their lives are most at risk. In 1996, about 21 percent of liver patients transplanted were Status 1 and about 30 percent were Status 2. Almost 48 percent of transplanted patients were Status 3, and less than 1 percent were Status 4.

The counter argument to this criticism is that, if sickest patients are always given preference, there is a less efficient use of the available livers, because the sickest patients (Status 1) have lower survival rates than transplant recipients with other statuses. Others say that if less sick patients receive lower preference than under the current policy, more of them will become sicker while waiting and then will have lower survival rates when they are eventually transplanted. Optimally, patients should be transplanted at a time when they are sick enough to benefit from a transplant, but not so sick that the risk of losing the graft is heightened. OPTN data show, however, that at one year after transplant there is about an 11 percentage point difference in patient survival rates and 13 percentage point difference in graft survival rates between former Status 1 and 2. Some argue that part of this difference is due to a side effect of local preference rather than greater risk of graft loss: Status 1 patients, they assert, often get an inferior organ that was made available only after it was turned down for use for any patient in another local procurement area.

Table 6, taken from pages 143 and 149 of the 1997 Annual Report of the OPTN and Scientific

Registry shows graft and patient survival rates of liver transplant patients, by status:

Table 6
Three Month and One Year
Graft and Patient Survival Rates of Liver Transplant Patients by Status

Waiting List Status at Transplant	N	3 Month Survival Rate		One Year Survival Rate	
		Graft	Patient	Graft	Patient
Status 1	1,019	74.6 %	81.9 %	67.7 %	76.3%
Status 2	1,562	84.0 %	89.8%	77.1 %	83.6%
Status 3	3,437	90.0 %	95.1%	84.0 %	91.4%
Status 4	91	87.8 %	97.6%	82.2 %	93.7%
Unknown	162	n.c.	n.c.	n.c.	n.c.
Overall	6,271	85.4 %	91.6%	79.1 %	87.0%

Note: Covers patients transplanted 1994-95 for which a survival time could be determined. n.c.=not calculated

Another frequent criticism of the current policy is that there is wide variation in waiting times from one geographic area to another. A counter argument is that this variation cannot be attributed entirely to the allocation policy, because it may also be a function of patient selection decisions and the number of organs procured locally. However, the allocation policy, particularly as it relates to the size of the initial allocation area, is a major determinant of variation in waiting times. For livers, waiting time differentials among transplant hospitals and among organ allocation areas vary by a factor of five or more.

A third criticism of the "local first" policy is that it greatly limits patient choice. If some non-local transplant hospitals do a better job and attract more patients, these patients come to those hospitals only at the price of a reduced chance for a transplant and compete with each other for the limited supply of organs

available locally. A counter argument is that some patients prefer to list at local hospitals and that an assured supply of local organs facilitates this particular choice.

Consideration of Alternative Policies Following discussions with the Department, which suggested that computer modeling be undertaken, UNOS contracted with the Pritsker Corporation in 1995 to develop a computer simulation model for liver allocation. The model presents the hypothetical outcomes resulting from the application of a number of alternative allocation policies. Among the many outcomes measured were: patients transplanted, percentages of patients transplanted by status, number of pre- and post-transplant deaths, median waiting times, and distance from donor location to transplant location.

The Liver/Intestinal Transplantation Committee of the OPTN considered seven policies that were most representative of all those modeled, including a policy for national sharing proposed by the University of Pittsburgh Medical Center (UPMC). The UPMC proposal and the other options had also been modeled by the CONSAD Research Corporation under contract with the UPMC. The Committee's subsequent recommendations were reviewed by the OPTN Patient Affairs Committee and by its Allocation Advisory Committee which put forth an alternate proposal. This proposal included a modest component of regional sharing of organs, but rejected major regional sharing as well as the national sharing advocated by UPMC.

At its meeting in June 1996, the Board of Directors considered the policies proposed by the Liver/Intestinal Committee and the Allocation Advisory Committee, as well as the existing liver allocation policy. The Board decided to change the existing policy in several ways, including redefining Status 1 to include only patients with "acute" failure, placing other patients in intensive care into the broader Status 2 group along with other patients of lesser urgency, eliminating Status 4 as an urgency category for

prioritizing liver transplant candidates, and mandating regional rather than local sharing for the newly defined Status 1 group (region for Status 1 allocation would be the area encompassing the 20 percent of the total number of Status 1 and 2 candidates on the national list who are nearest to the available organ). The Board of Directors then sent this proposal into an OPTN public hearing process held in the fall. In November 1996, the Board voted to adopt the new Status definitions, but to drop regional sharing. This change was scheduled to take place in January 1997. However, for the reasons described below, the Board suspended the new Status definitions (except for dropping Status 4) and the previous allocation system remained in place with little change.

At the Department's public hearing in December 1996, these system revisions became a major issue. The *de facto* effect of the Board's vote, as presented by many witnesses and uncontradicted by any evidence, was substantially to disadvantage the group called "chronic crashers", which had previously had a high priority as the predominant group within Status 1. In effect, the Board had increased the priority for "acute" patients with high medical urgency and little waiting time at the expense of another group with almost equally high medical urgency. While the Board did not present a formal rationale for the change in the record of its meeting, the change appears to be premised on the Board's belief that acute patients have a higher survival rate if transplanted promptly, and were disadvantaged under the current system, as well as its belief that some types of chronic liver disease, for example liver disease caused by alcoholism (alcoholic liver disease or ALD), had substantially lower survival rates.

As to the survival rate issue, the Department agrees with the approach taken by the American Medical Association in its report that supported the 1996 Code of Medical Ethics provisions discussed earlier. The report noted, "only very substantial differences in the likelihood of benefit among patients are relevant to allocation decisions." In fact, as reported in the UNOS Update magazine of September/October

1996, the “acute” category of fulminant liver failure actually has a lower survival rate after transplant than most types of chronic liver disease.

With respect to ALD, the Department notes that data presented at a National Institutes of Health Workshop indicated, “[r]ates of graft and patient survival after liver transplant for ALD are excellent and are similar to those for other chronic liver diseases....”

As a result of the airing of these matters at the HHS hearing, the OPTN Board of Directors rescinded its decision and placed the new policy on hold (while allowing, however, limited experimentation with broader sharing for “acute” patients in two OPTN regions). The net effect was temporarily to restore the prior system. At its meeting of June 25-26, 1997, the OPTN Board approved another policy, which would favor “acute” over “chronic crasher” patients. This revised policy puts the “acute” group first, the “chronic crasher” group second, and less urgent patients lower. Whatever the merits of giving preference to “acute” or “chronic” patients, these changes do little to reduce the fundamental inequities affecting patients across the country, the vast majority of whom have “chronic” liver disease. On the other hand, the new preference for “acute” patients exhibits a commendable understanding of the crucial argument in favor of this group: medical urgency.

All of these policy priorities, ranging from STAT to “acute”, represent OPTN attempts to favor the most urgent needs. In its performance goals, the Department retains and emphasizes this recurring theme of OPTN policies regarding allocation of livers as well as other organs.

In light of the extensive deliberative process within the OPTN, the many policies that have been considered, the substantial technical information available, the availability of two modeling tools that provide approximate quantitative estimates of the differing effects of alternative policies, and above all the demonstrated inequity of the current liver allocation policies, the Department is not providing the OPTN

the same period of time to reform liver allocation policy that it is providing for other organs. For all organs other than livers, the OPTN has one year from the effective date of these regulations to develop and submit to the Department allocation policies that meet the aforementioned performance standards. For livers, the Secretary is allowing 60 days from the effective date of these regulations. The Secretary appreciates that this time is far shorter than normal OPTN time frames, which include an opportunity for public comment. However, lengthy deliberations have already occurred and a great deal of information is available that will facilitate rapid reform. Moreover, the regulation specifies that no further public comment need be solicited by the OPTN before the deadline, although the OPTN may choose to do so. Similarly, the OPTN may choose to begin this process immediately if it believes that more time is required.

The final rule requires that the OPTN submit proposed transition procedures at the same time that it submits the proposed new allocation policy, together with supporting data. The Department will review these materials expeditiously, along with alternative proposals and public comments. The Department's plan is to obtain public input immediately following the deadline for the OPTN proposal. Commenters may propose alterations or alternatives. We ask that all proposals, whether from the OPTN or commenters, identify likely effects on inequalities in waiting times for patients of like medical urgency, on mortality, on life-years, on likelihood of organ wastage, and on other outcomes of importance.

The Secretary anticipates that similar procedures will be followed for other organs. In assessing these reforms for both livers and other organs, the Secretary will take into account that increased donation, more objective listing standards, and objective medical criteria for status categories all have significant potential for reducing geographic inequities. However, the Secretary has seen no evidence suggesting that fundamental inequities can be removed in the near future without broader geographic sharing of organs.

This final rule has not established specific quantitative measures that an OPTN liver allocation policy must attain to receive Secretarial approval. We expect the OPTN to use its medical expertise and consultative process to develop an appropriate policy. However, based on the use of the performance goals as a regulatory framework, it is unlikely that the Secretary would approve a policy that did not achieve a significant reduction in the disparity of waiting times, particularly for the most urgent patients.

(d) Directed Donation (§121.8(e)) Proposed § 121.7(d) on directed donation elicited several comments. Suggestions were made to delete the section on the basis that it would be misconstrued, and to refine it to take into account varying State laws. One commenter said that it contradicts the intent of the National Organ Transplant Act, and another said that directed donation should be discouraged but not prohibited. The existing OPTN policy discourages directed donation to designated groups or classes of people, but permits directed donation to named individuals. This policy is consistent with provisions of the Uniform Anatomical Gift Act, a model law that has been adopted by all States. The Department has retained in the final rule the language of proposed § 121.7(d) permitting directed donation of organs to named individuals. See, § 121.8(e). It should be pointed out that the final rule permits directed donation of an organ to named individuals only.

8. § 121.9 - Designated Transplant Program Requirements

Section 1138 of the Social Security Act creates an extraordinarily severe sanction for failure to comply with approved OPTN rules and requirements. This, in turn, would make it unfair and impossible to create standards higher than a threshold that any competent hospital might attain. In the proposed rule, the Department suggested the idea of “designated transplant programs” as a way around this dilemma.

Under this approach, failure to meet certain OPTN standards could result in an inability to receive organs, without necessarily jeopardizing either other transplant programs at the same institution or all Medicare and Medicaid reimbursement. No commenters objected to this approach, and no controversy over this approach surfaced at the public hearing. Accordingly, the Department has decided to retain the proposed approach, while improving it to reflect useful suggestions from commenters.

Most of the commenters on this section of the proposed rule recommended that the standards for the training and experience of transplant surgeons and transplant physicians, required for designation under proposed § 121.8(a)(2), apply also to Medicare-approved transplant programs designated under proposed § 121.8(a)(1). Three commenters suggested that transplant programs be designated on the basis of a minimum volume of transplant procedures and on patient survival standards, criteria now used in approving certain transplant programs for reimbursement under Medicare. Another commenter said that the NPRM was contradictory in admitting as OPTN members all Medicare-approved transplant hospitals, while expressing concern about proliferation of transplant hospitals and emphasizing that the Department did not wish to exclude hospitals from entering the field of transplantation. In the preamble to the proposed rule, the Department stated that the criteria for designation under proposed § 121.8(a)(1) and (2) are complementary, providing designated transplant program status to programs that meet Medicare standards, as well as to non-Medicare-approved programs which meet other requirements established by the OPTN. The Department's concern about the number of transplant hospitals was expressed in the context of "uncontrolled proliferation of transplant facilities," that is, permitting designated status without a method of ensuring the quality of care. See, 59 FR 46488.

The Department sees the merit in having uniform standards for designated transplant programs, but believes that it would be disruptive to impose them unilaterally at this time. Instead, the Secretary will

consider this issue in the context of revising the OPTN and Medicare standards. In that light, the Department has asked the OPTN contractor to consider developing standards regarding risk-adjusted graft and patient survival rates, and possibly volume of transplant procedures, if the latest scientific evidence supports such standards. If appropriate, such standards could supplement the requirements for designated transplant programs under § 121.9, following the notice and comment provisions of the Administrative Procedure Act.

The OPTN contractor, UNOS, said that the OPTN would not be able to provide patients with information about key personnel in Medicare-approved transplant programs, because it would have such information only for transplant programs designated under proposed § 121.8(a)(2). In addition, UNOS suggested that the OPTN be given authority to collect, maintain, and distribute data on key personnel for all transplant programs. The Department believes that the OPTN should define such a role through its Board of Directors' policy development process under § 121.4, and has asked the contractor to do so. Thus, explicit regulatory language is not required. In the meantime, to the extent that information is not readily available from the OPTN, we expect individuals to obtain it from the transplant programs themselves.

Two commenters suggested that a conflict exists between proposed § 121.8 (c) and proposed § 121.3(d)(2) with respect to designation of transplant programs and membership of transplant hospitals. Under proposed § 121.3(d)(2), the OPTN is directed to accept as members of the OPTN transplant hospitals which meet the requirements of proposed § 121.3(c)(1) or (2). Under proposed § 121.8(c), (now § 121.9(c)), the OPTN may accept or reject applications from transplant programs for designated status. There is no conflict, because membership under § 121.3 does not confer designated status under § 121.9. One commenter said that proposed § 121.8(a) should indicate that designated transplant programs are also

OPTN members. The Department has edited that paragraph in accordance with the suggestion. See, § 121.9(a). We have also added to § 121.9(c) a requirement that the OPTN act "within 90 days" on requests for designated status, making it comparable to the change made in § 121.3(c)(3), discussed above.

With respect to the disciplines listed in proposed § 121.8(a)(2)(v) as areas for collaborative involvement for designated transplant programs, two commenters suggested adding histocompatibility and immunogenetics. The Department has done so. See, § 121.9(a)(2)(v). The commenters also suggested that the term "tissue typing" in proposed § 121.8(a)(2)(vi) be changed to "histocompatibility testing." The change has been made. See, § 121.9(a)(2)(vi).

The Department also has added a provision at § 121.9(a)(2) requiring transplant programs to have adequate resources to provide transplant services to their patients and promptly to notify the OPTN and patients listed for transplantation if the program becomes inactive. We are aware of at least one instance in which a transplant program became inactive, yet did not advise its patients of its inability to perform transplants. Such a situation also could lead to use of the enforcement provisions of § 121.10.

9. § 121.10 - Reviews, Evaluation, and Enforcement

Two comments were received on this section of the proposed rule. In response to one comment, an editorial suggestion, the Department has clarified proposed § 121.9(b)(1)(iii) to indicate that compliance by member OPOs and transplant hospitals with OPTN policies, as well as regulations, is covered in reviews and evaluations carried out by the OPTN. See, § 121.10(b)(1)(iii).

The other comment was an expression of concern about patients listed at transplant programs whose designated status to receive organs for transplantation may be suspended. The Department wishes to assure all who share this concern that the enforcement provisions of § 121.10(c) allow for an orderly phase-out and transition period should such a situation occur. Under § 121.10, the OPTN is required to

monitor the compliance of individual transplant programs, to report to the Secretary the results of any reviews or evaluations that indicate noncompliance, and to make recommendations for appropriate action by the Secretary. The Secretary expects the OPTN to pay particular attention to programs experiencing difficulty. The rule further permits the Secretary to request more information from the OPTN or from the alleged violator, or both, before accepting or rejecting the OPTN's recommendations, or to take any other action the Secretary deems necessary. We expect that enforcement of these provisions will follow the pattern established by UNOS and member transplant hospitals in seeking voluntary compliance with OPTN policies in the past. That is, through a dialogue between the OPTN (and the Secretary, if necessary) and the transplant hospital alleged to be in violation of the rules, every effort will be made to reach a resolution before a decision is made to suspend a transplant program's designated status. It is the Secretary's intention that the OPTN develop a policy which minimizes disruption and cost to patients, and keeps them informed. The best interests of patient care will be paramount in monitoring and enforcement of compliance with this rule. In this regard, we have also elaborated on the procedures for OPTN reviews of transplant hospitals and OPOs. The OPTN shall conduct those reviews in accordance with the schedule specified by the Secretary and shall report progress on those reviews to the Secretary. See § 121.10 (b)(3) and § 121.10(b)(4).

10. Proposed § 121.10 - Appeals of OPTN Policies and Procedures

The Department received two comments on this section of the proposed rule. One commenter pointed out that appeals submitted to the Secretary must be sufficiently clear and substantiated. We agree that the Secretary must have appropriate information on which to base a decision, and believe that the language of the proposed rule provides the latitude needed for the Secretary to obtain such information.

See, § 121.4(d). The other commenter expressed an opinion that the Secretary's role in approving policies and deciding appeals could lead to arbitrary and capricious actions, and suggested that the Secretary's decisions be published in the Federal Register. Similar points were raised in comments about proposed §§ 121.3 and 121.7 regarding publication of the Secretary's decisions on allocation and other policies of the OPTN, discussed above.

The Secretary's authority under proposed § 121.10(b) is not dependent on appeal and may be exercised at any time. We have moved the language of proposed § 121.10(a) to § 121.4(d). Because proposed § 121.10(b) is redundant in light of § 121.4(b)(2) and (d), we have deleted this section from the final rule.

11. § 121.11 - Record Maintenance and Reporting Requirements

Most of the comments on this section expressed concern that the proposed rule falls short of needed protections of confidentiality, and suggested as a model the protections delineated in MEDPAR, a Medicare data system used by HCFA. We agree with the need to ensure protection of confidentiality and believe that the protocols in MEDPAR may lend themselves appropriately to the records falling within the purview of § 121.11. We also believe, however, that the design of a system to protect the confidentiality of OPTN records should be left to the OPTN, subject to the Secretary's review and the data release provisions of this final rule. We expect the OPTN to submit for the Secretary's consideration a policy which will protect the confidentiality of OPTN records, but at the same time permit access by researchers

to the OPTN and Scientific Registry data bases. Thus, we have amended proposed § 121.11(a) to reflect that records must be maintained and made available subject to policies of the OPTN and this final rule, as well as to applicable limitations based on personal privacy. We have also amended this section from the original proposal to clarify that the OPTN must follow such standard practices as making its information transactions and dissemination electronic to the extent feasible (unless requested in hard copy), and in disseminating information to include manuals and other explanatory materials as necessary to assure that the material is easily and accurately understood and used. We have also emphasized in § 121.11(b) and elsewhere that the OPTN should use rapidly advancing Internet technology to make information swiftly, conveniently, and inexpensively available throughout the nation.

Two commenters suggested adding a requirement that member transplant hospitals submit data to the Scientific Registry, a repository of data on transplant recipients that is operated under contract with the Department. Proposed § 121.11(b)(1) requires that the OPTN submit data to the Scientific Registry. We agree that a parallel requirement for transplant hospitals and OPOs is also appropriate, and have added it. See, § 121.11(b)(2). Another commenter suggested establishing a 90-day time limit for the submission of data under proposed § 121.11(b)(2). Such an explicit provision is not necessary because proposed § 121.11(b)(2) requires that information be provided on a prescribed schedule. In addition, UNOS suggested requiring the submission of cost data to the OPTN. Although we believe the language of the proposed rule is broad enough to permit the OPTN to request submission of such data, we have added to the final rule the phrase "and other information that the Secretary deems appropriate." We have also corrected omissions in proposed § 121.11(b) by including the Secretary as a recipient of the information. We have added to the reporting requirements the phrase "the OPTN and the Scientific Registry as appropriate...." This reflects the fact that some data which are to be reported or otherwise made available

to the public are held by the contractor operating the Scientific Registry, while other data are held by the OPTN contractor.

The OPTN and the Scientific Registry are often asked by researchers, payers, the press, patients, and others for data. We appreciate the importance of the contractors' obligation to maintain the confidentiality of patient-identified data. However, we also recognize that data, collected as a consequence of Federally funded contracts and of official designation as a contractor of the Federal government, generally should be in the public domain. Even patient-identified data can be shared with researchers who provide appropriate protections against redisclosure. It is vitally important that *bona fide* researchers and modelers have ready and timely access to detailed data in order to explore ways to improve organ transplantation and allocation. Therefore, information should be made available to the public while protecting patient confidentiality. To correct the oversight of omitting this activity from the proposed rule, we have added § 121.11(b)(1)(v) which requires the OPTN and the Scientific Registry to respond promptly (normally within 30 days) and favorably to requests from the public for data to be used for *bona fide* research or analysis purposes, to the extent that the contractors' resources permit, or as directed by the Secretary. The contractors may impose reasonable charges for responding to such requests. Pursuant to Federal government-wide policy under OMB Circular No. A-130, charges should reflect only the marginal cost of preparing the data for dissemination, not the cost of collecting or maintaining it.

We have also added language in paragraph § 121.11(b)(1)(vi) saying that the contractors must respond similarly to reasonable requests from the public. The regulation does not require the contractors to satisfy every request; however, the ability to charge for data requests should enable the contractors to accommodate most requests. In addition, the contractors would have to provide ready access to data that it originally received from transplant hospitals and OPOs, to these same institutions. See, §121.11(b)(1)(vii).

The Secretary has added language to § 121.11 (b)(2) making clear that hospitals and OPOs must provide data directly to the Department upon request, and must authorize the OPTN and Scientific Registry to release data to the Department or others as provided in the regulation. The OPTN has informed us of difficulties it has in complying with both instructions from the Department and its perceived obligation to these institutions not to disclose data that might be made public by the Department. While we do not believe this to be a serious dilemma, we have drafted the final rule to make it clear that any hospital or OPO must, as a condition of its OPTN membership, make data available without restriction for use by the OPTN, by the Scientific Registry, by the Department, and in many circumstances by others, for evaluation, research, patient information, and other important purposes. In this regard, we particularly emphasize that we are requiring that current, institution-specific performance data be made available so that patients, payers, referring physicians, the press, and others can appraise the quality of transplantation programs. The Congress made this an obligation of the OPTN.

We have added language in § 121.11(b) (1)(I)(B) stating that the OPTN and the Scientific Registry shall submit to the Secretary information the Secretary deems necessary to prepare the Report to Congress required by section 376 of the Act, in order to clarify the contractors' responsibility in this area.

To complete the articulation of this policy, we have added a new paragraph (c) to §121.11, "Public access to data." This paragraph provides that the Secretary may release to the public information upon determining that the release will serve the public interest. For example, data on comparative costs and outcomes at different transplant programs, information on waiting list time, and information on the frequency with which transplant hospitals refuse offers of organs for their listed patients, will assist patients and their families and advisors in deciding where they wish to be transplanted. This release of data is consistent with section 375 of the Act, 42 U.S.C. 274c, which directs the Department to provide information to patients, their families, and their physicians about transplantation resources and about the

comparative costs and patient outcomes at each transplant hospital affiliated with the OPTN, in particular. It is also consistent with the Department's practice of having the contractor include in its published reports extensive data, including transplant hospital-specific survival data.

The provisions of §121.11(c) were not included in the NPRM of September 8, 1994. To delay the implementation of this paragraph would be contrary to the public interest in that the decision-making of these parties regarding this life-saving procedure should be fully informed as soon as possible. The release of data is essential to allow patients, their families, and their physicians to make the most informed decisions possible about transplantation. Furthermore, the release of these data is consistent with the above-cited section of law and with the well-established practice of publishing center-specific outcome data, and thus public comment prior to publication is unnecessary.

The Secretary specifically requests comments on whether the above provisions sufficiently achieve the several important purposes served by provision of information to the OPTN, the Department, and the public, while protecting patient privacy.

12. §121.12 - Preemption

A new section regarding preemption has been added to the final rule. This section does not require notice and comment rulemaking by the agency, as it does not alter the rights and responsibilities of any party. Instead, it simply applies the preemption principles derived from the Supremacy Clause of the United States Constitution. The Secretary is directed by section 372 to oversee a national system for distribution of organs, and the policies of the OPTN currently require organ sharing across State lines. The performance goals and indicators articulated by these rules are almost certain to increase interstate sharing.

At least one State has passed a law that appears to limit organ sharing policies. A national organ sharing system based primarily on medical need, with geographic considerations having less weight than at present as an allocation criterion, would be thwarted if a State required that, prior to sharing an organ with any other State, there be a written agreement with that other State or a requirement that the hospital or OPO first attempt to match the organ with an eligible transplant candidate within the State, regardless of status.

Similarly, a State enforcing such a law would almost certainly render impossible the compliance of transplant hospitals and OPOs within that State with rules and requirements of the OPTN, and thus would jeopardize their ability to obtain Medicare and Medicaid reimbursement. This too would thwart the Federal scheme created by Congress.

A further negative effect would flow from the enactment by additional States of such restrictive laws. If more States were to enact such laws, greater disruption in the allocation of organs under the OPTN's policies would occur. Patients registered for transplants in such States would almost certainly die as a result of the restrictions on organ sharing, while other patients would receive organs even though their transplants would not be approved until later under the OPTN's policies. Accordingly, for policy as well as legal reasons, the Department has added the preemption statement to the regulation.

The preceding discussion constitutes a Federalism Assessment, as required by Executive Order 12612, and we certify that this rule was assessed in light of the principles, criteria, and requirements of that Order.

III. Economic and Regulatory Impact

A. Legal Requirements

A number of statutes and executive orders require us to analyze the economic impacts of final rules.

Executive Order (E.O.)12866 requires that all regulations reflect consideration of alternatives, of costs, of benefits, of incentives, of equity, and of available information. Regulations must meet certain standards, such as avoiding unnecessary burden. Special analysis is required for regulations which are "significant" because they create economic effects of \$100 million or more; create adverse effects on the economy, public health, or other named categories; create serious inconsistency with actions of another agency; or materially alter the budgetary impact of entitlements and other programs or the rights and obligations of recipients thereof; or raise novel legal or policy issues.

The Regulatory Flexibility Act requires that we analyze regulations to determine whether they create a significant impact on a substantial number of small entities (for purposes of the Act, all not-for-profit hospitals and all OPOs are categorized as small entities), and if so to prepare a Regulatory Flexibility Analysis exploring ways to mitigate adverse impact.

Executive Orders 12875 and 12612 (dealing, respectively, with "Enhancing the Intergovernmental Partnership" and "Federalism") require that we review regulations to determine if they unduly burden States, localities, or Indian tribes, or if they inappropriately infringe upon the powers and responsibilities of States.

Section 202 of the Unfunded Mandates Reform Act of 1995 requires that we determine whether regulations may result in the expenditure of \$100 million either by State, local, and tribal governments, or by the private sector.

The Congressional review procedure of section 801(a)(2)(A) of title 5, United States Code, enacted in 1996, requires that rules with an economic effect of \$100 million or more or other comparable

effects be classified as “major”, and that these rules may not take effect until the Congress has had 60 days to review them.

We have determined that this rule will not have consequential effects on States, local governments, or tribal governments, because it affects primarily the operation of private sector OPTN functions and the allocation of organs among patients based on their medical condition. It will not require an expenditure of \$100 million or more by the private sector. Therefore, it does not meet the special consultative requirements of the Unfunded Mandates Reform Act. We have determined that it will not have a significant impact on a substantial number of small entities, and so certify under the provisions of the Regulatory Flexibility Act. However, because there is significant concern over the effects of changes in allocation policies on smaller hospitals, and because we considered as an alternative the possibility of imposing quality standards on transplant hospitals, we have prepared a voluntary Regulatory Flexibility Analysis (RFA). The analysis which follows, together with the remainder of this preamble, constitutes an RFA. We have also determined that this is an economically “significant” rule under E.O. 12866 and a “major” rule for purposes of Congressional review of agency rulemaking. (This rule is also “significant” under E.O. 12866 because it “materially alters” the rights of recipients--patients--of entitlement and grant programs). The analysis that follows, together with the remainder of this preamble, constitutes a Regulatory Impact Analysis (RIA) meeting these requirements.

This combined Regulatory Impact Analysis and Regulatory Flexibility Analysis also serves to analyze the effects of policies that we expect to approve under the procedures put in place under this rule, and that are assessed in this preamble, including all organ allocation policies necessary to implement the performance goals and indicators that we establish.

At the time of the proposed rule, we stated that it would be premature to analyze alternatives because of the procedural emphasis of the NPRM. We stated that we would analyze comparatively the

range of options that we considered, including the existing OPTN policies, based on the comments and information we later received. Subsequent events explained earlier in this preamble, and the information that we have subsequently received, have made it both desirable and possible to analyze qualitatively, and in part to quantify, the effects of the substantive, non-procedural policies promulgated under this final rule. We are far better able to quantify the effects of changes in liver allocation policy than of changes in allocation policy for other organs. However, we expect those changes to be qualitatively similar, and this analysis covers all allocation policies.

B. Effects of Organ Transplantation

Industry Structure and Size. As indicated in Table 7 below, covering selected organs, transplantation services are a very substantial set of medical procedures, although only a very small fraction of the trillion dollar health care sector.

Table 7
Estimated Billed Charges for Transplants, 1996

Major Organ	No. Programs 1996	No. Transplants 1996	Average Billed Charges per Transplant 1996 (\$1000s)	Total Program Billed Charges 1996 (\$1000s)	Average Program Billed Charges 1996 (\$1000s)
Kidney	253	11,099	\$94	\$1,043,306	\$4,124
Liver	120	4,058	\$290	\$1,176,820	\$9,807
Pancreas	120	1,022	\$110	\$112,420	\$937
Heart	166	2,342	\$228	\$533,976	\$3,217
Lung	94	805	\$241	\$194,005	\$2,064
TOTAL PROGRAMS	753	19,366		\$3,060,527	
TOTAL HOSPITALS	281	19,366		\$3,060,527	\$10,892

Sources: Data on numbers of programs and hospitals 1996 Annual Report of the OPTN, page 20 and C-2. Data on transplants performed from Facts About Transplantation in the U.S., UNOS, July 23, 1997. Data on billed charges per transplant from "Cost Implications of Human Organ and Tissue Transplantations, an Update: 1996," by Richard H. Hauboldt, F.S.A., of Milliman & Robertson, page 30, excluding OPO charges.

These data show that on average, transplant programs generate revenues in the millions of dollars. Since most transplant hospitals operate several programs, the unduplicated revenue average across the 281 transplant hospitals that are OPTN members is about \$11 million annually. This includes not just the cost of the transplant procedure itself, but also pre- and post-transplant charges such as time in the hospital waiting for a transplant. Because the source of these data uses billed rather than negotiated charges, actual receipts may be somewhat lower than shown above.

The range of revenues is much broader than these averages convey because the number of transplants performed varies so widely. Table 8 below, taken from OPTN and Scientific Registry data, shows the dozen highest volume programs for liver transplants performed in 1995 and 1996. These dozen programs performed one fourth of all liver transplants. Taken together, the two dozen lowest volume programs of those that performed transplants in 1996 only performed about 80 transplants, 2 percent of the total. Among active liver programs, the median program performed about 30 transplants, while the average was about 36.

Table 8

12 of the Highest Volume Liver Transplant Programs, 1995-1996

Transplant Program	1995 Volume	1996 Volume
UCLA Hospital Center, Los Angeles, CA	230	245
Presbyterian-University Hospital, Pittsburgh, PA	209	179
Mount Sinai Medical Center, New York, NY	209	180
Jackson Memorial Hospital, Miami, FL	194	179
Baylor University Medical Center, Dallas, TX	140	118
University of Chicago Medical Center, Chicago, IL	132	130
University of California, San Francisco, CA	106	100
University of Nebraska Medical Center, Omaha, NE	94	81
Rochester Methodist Hospital, Rochester, MN	91	89
University of Alabama Hospital, Birmingham, AL	82	86
Shands Teaching Hospital & Clinics, Gainesville, FL	81	102
University of Michigan Hospital, Ann Arbor, MI	78	59
TOTAL	1,646	1,548

Source: 1997 Annual Report of the OPTN, pp. 391-396

Thus transplant volumes, and revenues, are highly skewed, with the average much higher than the median.

The billing cost data in Table 7 focus primarily on hospitals, and do not include procurement charges, which average approximately \$24,000 per major organ in 1996, for a total of approximately one-half billion dollars per year in addition to the \$3 billion spent at transplant hospitals. Procurement charges are paid through organ procurement organizations. OPOs are by law given local (in some cases state-wide or larger) monopolies through a review and designation system administered directly by the Federal government. Currently, there are 63 of them, averaging some \$8 million annually in revenues. Most of

the revenues of both transplant programs and OPOs are paid by Federally funded health programs, primarily Medicare and Medicaid, but also Federal Employees Health Benefits Program (FEHBP), CHAMPUS, the Uniformed Services and the VA. In total, the government is by far the largest single payer for transplantation.

Included in the data above, but not separately identified, are laboratory costs. These can be very substantial, as a wide range of condition-related tests are necessary to monitor patient urgency, and both donors and recipients must have a broad range of laboratory tests.

The data above also include follow-up charges for one year, but not subsequent follow-up charges for immunosuppressive therapy and other costs. These average, according to Milliman & Robertson, about \$7,000 for pancreas, \$16,000 for kidneys, and between \$21,000 and \$29,000 for the other major organs in 1996. Adjusted for survival, Milliman & Roberts estimate the five-year cost of major organ transplants including follow-up costs as follows: heart, \$317,000; liver, \$394,00; kidney, \$172,000; lung, \$312,000; and pancreas, \$149,000.

There are other sources of data on these categories of costs, each using somewhat different estimating techniques. Their estimates are generally comparable though sometimes lower. We note that such figures do not generally estimate the marginal cost of transplantation, after subtracting other costs that would be incurred if the patient did not receive an organ. Marginal costs are much lower. In the case of kidneys, a number of studies have estimated that transplantation costs are more than offset by reductions in other medical costs such as dialysis costs.

For purposes of the Regulatory Flexibility Act, an entity is considered "small" if it has revenues below a certain size threshold, or operates as a not-for-profit entity that is not dominant in its field. For health care providers, such as hospitals, the threshold amount is \$5 million in annual revenues. Taking

into account total hospital revenues and not just transplant revenues, few or no transplant hospitals fall below this threshold. However, the great majority of these institutions are not-for-profit entities, and hence qualify as “small entities” despite their substantial revenues.

Patient Effects. Table 9 below provides dramatic evidence of the importance both of increasing organ donation and of reducing unnecessary deaths while waiting for organs. Unlike growth in the waiting list, which in part reflects factors such as earlier and more aggressive listing, these data on deaths while waiting for organs provide clear evidence of the need for transplantation.

Table 9
Reported Deaths on the Waiting List
1988-1996

Organ	Year								
	1988	1989	1990	1991	1992	1993	1994	1995	1996
Kidney	739	759	917	975	1052	1285	1361	1510	1814
Kidney-Pancreas	0	0	0	0	15	61	71	86	91
Pancreas	6	23	21	37	33	3	13	4	5
Liver	195	284	316	435	495	562	657	799	954
Heart	494	518	612	779	780	763	724	769	746
Heart-Lung	61	77	68	45	44	51	48	28	48
Lung	16	38	50	139	219	252	286	340	385
Intestine	0	0	0	0	0	3	15	19	22
Overall	1,502	1,666	1,962	2,360	2,580	2,902	3,055	3,421	4,065

Source: UNOS web site at http://www.UNOS.org/sta_dol.htm, data as of January 13, 1997

The approximately 20,000 annual transplants of major organs fall into two broad groups. More than half are kidneys. In the case of kidneys, dialysis is an alternative to transplantation for extended periods of time. Therefore, for most patients transplantation is not a matter of immediate survival. Instead, the benefits of transplantation fall largely (though not exclusively) in the domain of improved quality of life. These improvements can be very substantial, as physical health while on dialysis is significantly impaired, and dialysis imposes major stresses and substantial inconveniences in carrying out normal activities. In sum, dialysis sustains life but not well-being whereas a transplant can and often does restore well-being. For other organs, a transplant is in most cases a matter of survival. There are life-prolonging technologies that work for some patients (e.g., left ventricular assist devices for hearts) but for most awaiting extrarenal organs, a transplant is literally essential to survival. Thus, in round numbers the annual benefits of organ transplantation include about eleven thousand lives vastly improved by kidney transplantation, and another eight thousand lives both vastly improved and prolonged by transplantation of other major organs.

It is common, in benefit cost analysis, to use a concept termed "value of a statistical life" to estimate in monetary terms the benefits from lives saved. Estimates of this value can be derived from information on the preferences of individuals for reduction in the risk of death, and their willingness to pay for such reductions. In this case, however, it is important to take into account two major factors that reduce the usefulness of a statistical life as a measure: (a) most organ transplant recipients are much older than average and hence gain fewer years than would average beneficiaries of other life-saving interventions, and (b) an organ transplant carries a substantial risk of either the graft or the patient not surviving. For example, according to historical data from the 1997 Annual Report of the OPTN (page 23), only 62 percent of cadaveric kidney grafts survive 5 years, and only 81 percent of these patients survive 5

years (patient survival is substantially higher because dialysis is usually an option if the organ fails). Five year patient survival rates for livers 72 percent, for hearts 67 percent, and for lungs 43 percent. As each year passes, additional patients die, though at lower rates than in the first year or two. Survival rates have improved in recent years, but the statistical expectation of increased longevity and/or graft survival from a transplant is on the order of a dozen years (a rough estimate since we do not yet know what the long-term experience will become), not the 40 years (half a lifetime) that underlies most estimates of statistical lives. Using the more conservative concept of a "statistical life-year" saved, then, the benefit from each year's cohort of approximately eight thousand non-renal transplant recipients approximates one hundred thousand life years. In a recent rule-making on tobacco, HHS estimated the value of a statistical life-year at about \$116,000 (see Federal Register of August 28, 1996, at page 44576). This was a conservative estimate that would reasonably apply to organ transplantation (though a figure several times as high could equally reasonably be used). Applying the conservative \$116,000 value to statistical life-years saved by non-renal organ transplants, the social benefit from each annual cohort of recipients is on the order of \$12 billion. (Additional benefits could be calculated for quality of life improvements for kidney recipients.) Thus, whether one counts lives saved, life-years extended, or improved quality of life, and whether or not expressed as dollars, the social benefits of transplantation far exceed the admittedly expensive costs of transplantation.

C. Effects of this rule

This rule creates three major effects. First, it establishes terms of public oversight and accountability for the entire organ transplantation system, and the OPTN in particular. We believe that this reform creates major public benefits in the categories of "good government," preserving public trust

and confidence in organ allocation, and assuring the rule of law. The Secretary does not believe that such oversight creates any consequential costs. Its benefits are substantial, but intangible. They may well lie primarily in future problems avoided (e.g., reduction in organ donation if the public were to lose confidence in the fairness of the OPTN in allocating organs) rather than in specific current problems solved.

Second, this rule requires creation of a system of patient-oriented information on transplant program performance. At present, the fundamentals of such a system exist through the efforts of the OPTN. The OPTN collects, validates, and analyzes a great deal of important information. It publishes, in collaboration with this Department, a Report of Center Specific Graft and Patient Survival Rates. This report consists of 9 volumes and 3,200 pages, and contains valuable information. However, from a patient perspective it is not up-to-date or easy to use. The most recent version was the 1997 report, but the data were current only up through April, 1994. The primary limitations of the Report are that the survival rates are for patients transplanted several years earlier and that there is no information regarding the waiting list at individual transplant centers. We believe the data should be more current. In addition, we believe center specific waiting times and numbers and percentages of transplant center organ turndowns of organs for non-medical reasons should be made available to the patients. Finally, versions are needed that are easy to use for patients, physicians, and families who wish to compare center performance on any or all of these dimensions.

Third, this rule will improve equity by creating performance goals against which the OPTN can reform current allocation policies. Such a reform has important benefits--though benefits virtually impossible to quantify--in their own right. We note that "equity" is an important goal under Executive

Order 12866. Unfortunately, improved equity is an extraordinarily difficult concept to quantify. It is a goal and as it is achieved, benefits accrue to members of society at large, to donor families, to transplant candidates, and to transplant recipients. We do have some measures of additional benefits arising in part from improved equity, such as life-years saved, but these are a separate category of benefit. We believe that a system that allocates organs to those most in need in accordance with sound medical judgment, but with as little regard to geography as reasonable, has profound benefits quite apart from those that are life saving.

Table 10 below summarizes a number of measures of the effects of alternative approaches to improved equity in organ allocation, for livers. Comparable data are not readily available for other organs, and for a number of reasons liver transplants are particularly susceptible to improvement (hearts, for example, are already shared regionally and kidney patients have dialysis options). However, these liver data suggest the kinds of improvements that can be made for other organs.

Table 10
Summary of Measures of Alternative Approaches to Liver Allocation

	1996 Policy	Allocation Committee	Inpatient First	National
Percent Transplanted by Hospitalization:				
Inpatient	59%	73%	96%	97%
Outpatient	41%	27%	4%	3%
Share of Organs:				
Local	78%	44%	38%	20%
Regional	18%	28%	31%	6%
National	4%	28%	31%	74%
Number Transplants:				
Initial	10,992	10,998	10,451	10,231
Repeat	1,663	1,659	2,189	2,425
Total	12,655	12,657	12,640	12,656
Number on Waiting List at End:				
	11,534	11,788	12,729	13,050
One Year Survival Rate:				
	80%	81%	76%	73%
Deaths:				
Pre-transplant	3,704	3,599	3,168	2,963
Post-transplant	2,539	2,555	2,967	3,144
Total	6,243	6,154	6,135	6,107
Life-years:				
Pre-transplant	26,600	27,193	29,443	29,915
Post-transplant	24,712	24,840	22,759	21,765
Total	51,312	52,033	52,202	51,680

Source: These estimates all come from modeling runs created by the Pritsker Corporation for the OPTN. Most of those results were included in information provided at OPTN Board of Directors meetings. All data cover a three year period, and are not annual estimates. Actual data for 1996 do not necessarily agree with these modeling estimates, which apply to future years.

These data show, in broad outline, the effects of several alternative policies for liver allocation. We emphasize that none of the alternatives modeled included the effects of improved listing and status standards, and for that and other reasons discussed below, these results cannot be taken as precise predictions of the effects of changes.

These data also omit a large number of alternative policies that have been modeled, in the interest of economy of presentation. Of particular interest are a set of policies that deal with a family of options that have been termed "time and distance weighted." This family of options seeks to minimize transportation of organs while achieving equity based on medical urgency and waiting time. In effect, organs are transported long distances only when there is no alternative for patients with high priority. Organs are kept locally when only very small differences in patient benefit could be achieved by regional or national transportation. Depending on the precise weights given to medical status, waiting time, and distance, inequities due to waiting time disparities can be greatly reduced. (See testimony of Dr. John P. Roberts of the University of California, San Francisco, presented at the public hearing and two letters from Dr. Roberts included as Exhibit L in the Liver and Intestinal Organ Transplantation Committee Report presented to the OPTN Board of Directors for its meeting on June 25, 1997) .

In Table 10, some of the most studied options are presented. These options focus increasingly on broader geographical sharing, and on greater reliance on medical urgency, from left to right. The first column simply presents the predicted results of 1996 policy. The "Allocation Committee" column shows the results of an option reviewed and subsequently rejected by the OPTN Board in 1996, that would have allocated organs to Status 1 (most urgent) patients across regions comprising 20 percent of the eligible hospitalized patients. Other patients would have received either a slightly improved or no chance at organs from out of the local area. Thus, this represents a very modest change towards regional sharing from current policy. The third column, "Inpatient First", shows the results of an option that would have allocated

organs first nationally to hospitalized patients, and only then to Status 3 patients. The “National” column shows the results of an option proposed by the University of Pittsburgh Medical Center that would have allocated organs by status, primarily on a national basis, from most to least urgent (even the “National” proposal preserved a substantial role for local allocation, by allocating first to a local patient in Status 1, then nationally, then to a local patient in Status 2, then nationally, etc.).

One very striking result is that even a modest policy change can very substantially change the kinds and places of patients receiving organs. The Allocation Committee option decreases the share of livers allocated to non-hospitalized patients (Status 3 and 4) from 41 percent to 27 percent, and decreases the number of organs shared locally from 78 percent to 44 percent.

Taking the remainder of the rows in order, broader sharing has no consequential effect on the number of transplants, but raises the number of repeat transplants, thereby reducing the number of individuals transplanted. This is a consequence of transplanting very sick patients who are more likely to reject an organ graft after transplantation. The number on the waiting list rises when organs go first to more urgent patients. This is both a good and bad outcome--longer waiting is “bad” but not if the alternative for other patients is death. Survival rates decrease with a priority to the most urgent because the most urgent patients tend to have more advanced disease and additional co-morbidities (as discussed below, we do not believe that current simulation results accurately measure likely survival rates). However, as shown in the estimate of deaths, the net effect of these changes is to reduce premature death, despite the decrease in survival rates. Of importance is that the net total change in deaths masks a very pronounced difference in direction for deaths pre-transplant (which are substantially reduced), and deaths post-transplant (which in the Pritsker model increase almost enough to offset pre-transplant lives saved--but see discussion below of the CONSAD model). Life-years exhibit a similar pattern to deaths, but are arguably a better measure of real effects. Over a longer period of years, the total number of people dying under all

options will approach equality--but only if there is no increase in transplant survival rates through medical progress. But a life-year lived is never "lost" and represents an unambiguous gain for the patients who benefit. Unfortunately, the post-transplant life-years increase very little or decrease under broader sharing (as estimated by Pritsker), whereas the years on the waiting list, not dying but not well, increase dramatically.

As shown both in the Pritsker results and in the CONSAD results presented below, no organ allocation gains are free. Taking as an example deaths under a National policy, the Pritsker model estimates that over a three year period some 700 fewer people would die pre-transplant, and some 600 more people would die post-transplant. These are changes of one-fifth or more in the number dying in each group. Both costs and benefits are very high, thus reducing the net benefit substantially.

The CONSAD model produces generally similar results, but shows a distinct difference in the magnitude of deaths and life-years (as shown in Table 11):

Table 11
 Numbers of Pre- and Post-Transplant Deaths and Life Years
 Under Alternative Liver Allocation Policies

	1996 Policy	Allocation Committee	Inpatient First	National
Deaths:				
Pre-transplant	4,571	4,394	4,060	4,216
Post-transplant	2,468	2,487	2,734	2,527
Total	7,039	6,881	6,794	6,743
Life-years:				
Pre-transplant	15,093	17,837	19,580	18,683
Post-transplant	38,107	38,096	35,537	36,465
Total	51,200	53,933	55,117	55,148

Source: CONSAD model run dated March 24, 1997.

As shown, under the CONSAD model the net life saving and life-year saving effects of broader sharing are much more pronounced, as well as more favorable to post-transplant experience. CONSAD shows National allocation preventing a net of over 300 deaths and saving a net of almost 4,000 life-years, in contrast to Pritsker's estimate of about 140 deaths and about 400 life-years (though 900 life-years for Inpatient First). These are not small differences. Under the Pritsker model, deaths would decrease, and life-years would rise, only about 2 percent from current levels under the most favorable result for broader sharing. Under the CONSAD model, deaths would decrease about 4 percent and life-years would rise about 8 percent. Realistically, in view of the modeling issues discussed below, a 2 percent difference may represent less than the possible error in the model, though an 8 percent difference is much more robust--if the model parameters and assumptions are accurate. But even the CONSAD results indicate that improved

allocation policies have at best a limited potential to improve outcomes. In contrast, improved organ donation represents an unambiguous and potentially much larger gain.

There are known differences in model assumptions and approaches that illustrate the strengths and weakness of both efforts. The Pritsker model results “throw away” the first of the four years modeled, to show more clearly the long-term rather than transitional effect of change. In contrast, the CONSAD model cumulates the results of years one, two, and three, rather than two, three, and four. Since many life-years and deaths occur in the transition year, totals vary for this reason. Second, the Pritsker model assumes that all transplant programs operate at the same effectiveness as in the early 1990's, all through the modeling years. The CONSAD model, in contrast, assumes a slow but steady increase in transplant program performance and patient survival. This assumption naturally results in fewer deaths and more life-years gained in CONSAD runs, differentially in favor of those who would otherwise die but could now expect to survive.

One difficulty shared by both models is that the OPTN has not released current data on transplant outcomes. Thus, these modeling results rely on data centering around 1990 and 1991 (including several years before and after) rather than on the latest outcome data. Because current graft and patient survival rates are known to be higher, this makes certain outputs, particularly graft survival rates, deaths, and life-years, inaccurate. CONSAD attempts to estimate recent progress, but this is not a complete substitute for better baseline data.

Showing the importance of progress over time, UNOS data show that between 1990 and 1995, one year patient survival for liver transplant recipients increased from 83 to 87 percent.

Neither model completely captures a variety of real world nuances. For example, under current policies survival rates for the sickest patients who receive organs from outside their local area may be

influenced adversely by the sometimes lower quality of the organs they receive that have been turned down elsewhere. But no hard data exist, and neither model attempts to estimate such an effect. Neither model attempts to deal with a hypothetical breakthrough in technology. Neither model deals with the “friction” involved in transporting organs over broader geographic areas (although they do produce estimates of increased organ travel); both assume no wastage or reduced graft survival results. None of these differences or commonalities imply a fatal weakness in either or both of these models, but simply a recognition that simulation modeling is by its very nature a partial and incomplete attempt to predict results with any number of assumptions potentially affecting outcomes.

From the Department’s perspective, what is most important about these modeling results is that despite the somewhat different interests of their sponsors and the potential bias that might result, and the infant efforts that they represent, these two independent efforts agree almost completely on the qualitative effects to be expected from changes in allocation policies, and substantially on the magnitudes involved as well.

More complex to display are measures that capture likely effects of improved policies on disparities in waiting times. As discussed earlier in this preamble, program-specific, area-specific, and region-specific results look very different, because aggregation masks disparities. However, even regional differences are substantial. Table 12 below follows shows the disparities under the 1996 policy, the Allocation Committee (regional) proposal, the Inpatient First proposal, and the National (local first, then national) proposal, as measured in average days waiting for a liver transplant:

Table 12
Analysis Average Days Waiting for a Liver Transplant
under Alternative Liver Allocation Policies

OPTN Region	1996 Policy	Allocation Committee	Inpatient First	National
Region 1	102	123	110	105
Region 2	126	120	121	124
Region 3	23	70	81	109
Region 4	91	91	100	113
Region 5	121	113	109	119
Region 6	56	107	94	107
Region 7	118	113	105	110
Region 8	110	116	106	122
Region 9	119	99	107	115
Region 10	88	92	93	110
Region 11	70	76	88	123
Standard Deviation	32.24	17.93	11.55	6.81

Source: CONSAD model run dated March 24, 1997.

In this table, the standard deviation entry measures the extent to which regional averages differ. The standard deviation is a statistical measuring tool. In this context, it means that under the current system about two-thirds of the regions are within 32.24 days of the average (both longer and shorter), and the remaining one-third are more than that many days longer or shorter than the average. As these results show, even modest geographic sharing based on a proxy for medical need greatly reduces disparities in waiting time, from a standard deviation of 32.24 days under current policy to as few as 6.81 days under a national system of distribution. (Of course, as discussed previously, current measures of waiting time

disparities are weak because the lack of listing standards does not create uniform, status-related measures that would be truly fair as tie-breaking criteria.)

Another dimension of improved equity arises from reducing the role of ethically irrelevant characteristics such as race or insurance coverage in organ allocation. We already know, from prior studies, that racial minorities--particularly African Americans--may not benefit to the extent that their medical need warrants. In the final rule, as noted previously, we have tasked the OPTN to develop policies to reduce socio-economic inequities. No data from the modeling efforts or other sources enable us to predict precise effects, even if the full potential of such policies were clear. However, to the extent that improved allocation policies reduce the ability of patients, payers, or physicians to "game" the system, it will necessarily benefit the more disadvantaged patients.

The performance goals created by this rule do not directly mandate any of the allocation options just discussed. Instead, we require the OPTN to craft new policies that achieve those goals. To the extent that the modeling results capture our expectations, we expect those reformed policies to show results much more similar to the rightmost two columns in tables above than to the leftmost two columns. But neither precise policy nor expected results have been modeled yet. And neither modeling effort purports to measure directly equity, except insofar as reduced disparities in waiting time in status capture this goal.

One final effect of the Department's overall initiative is extremely important, though not attributable to this regulation. Increases in organ donation are an unambiguous benefit. If, as seems possible, the package of initiatives proposed by the Department could increase organ donation by 20 percent or more, the benefits in lives saved and life-years increased would both dwarf the estimates of these effects as calculated by the simulation models. Increased donation would also reduce waiting times.