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The OPTN/UNOS Policy Development Cycle: Challenges and Opportunities

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Abstract The policies governing organ procurement and transplantation in the USA are developed and implemented by the Organ Procurement and Transplantation Network (OPTN) which was established by the National Organ and Transplant Act in 1984. The OPTN, operated by the United Network for Organ Sharing (UNOS), develops and revises policies through an iterative, evidence-based, consensus-driven process involving input from OPTN/UNOS committees and regions, the donation and transplant community, and the general public. Nonetheless, circumstances have arisen where the OPTN/UNOS Board of Directors has acted prior to public comment in response to urgent situations, including development of new technology, adoption of new statutes and regulations, and in response to legal challenges. These events have led to updates to the OPTN bylaws and processes to provide a more transparent framework for future actions and improve the efficiency of the OPTN policy development process. Nonetheless, opportunities for improvement remain, particularly with regard to alignment of OPTN policies with those developed by the Centers for Medicare and Medicaid Services.

Keywords Organ transplantation · Organ procurement · Organ allocation · Public policy

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Introduction

The policies governing organ procurement and transplantation in the USA are developed and implemented by the Organ Procurement and Transplantation Network (OPTN). Originally established by the National Organ and Transplant Act (NOTA) [1] in 1984, the OPTN is operated by a non-profit contractor to the Division of Transplantation (DOT) within the Health Resources and Services Administration (HRSA) of the US Department of Health and Human Services (HHS). The United Network for Organ Sharing (UNOS) was awarded the initial contract in 1986 and has held it since that time.

NOTA established the legal foundation for the regulatory framework for the OPTN. In the initial version of NOTA, direction regarding allocation policy was limited to a brief section directing the OPTN to establish a waiting list (Table 1). NOTA was subsequently amended to include directives to establish membership and allocation criteria, seek public comment (PC) regarding these criteria, recognizing the unique healthcare needs of children and carrying out studies to improve organ procurement and allocation including increasing transplantation among special populations (Table 1).

The regulatory framework for the OPTN, including detailed criteria for the development of organ allocation policy and oversight by the HHS secretary, was further established when the Final Rule governing the OPTN was implemented in 2000 [2]. The Final Rule directs the OPTN to develop policies for equitable allocation of deceased donor organs, policies for testing of organ donors and recipients to prevent the spread of infectious diseases, and policies to reduce inequities resulting from socioeconomic status.

The Final Rule also provides explicit guidance regarding allocation policy, including establishing minimum listing criteria, developing organ allocation policies based on sound

Table 1 National Organ Transplant Act provisions for the OPTN

The Organ Procurement and Transplantation Network shall
(A)[a] Establish in one location or through regional centers—
(i) A national list of individuals who need organs and
(ii) A national system, through the use of computers and in accordance with established medical criteria, to match organs and individuals included in the list, especially individuals whose immune system makes it difficult for them to receive organs;
(B) Establish membership criteria and medical criteria for allocating organs and provide to members of the public an opportunity to comment with respect to such criteria;
(C)[a] Maintain a 24-h telephone service to facilitate matching organs with individuals included in the list;
(D) Assist organ procurement organizations in the nationwide distribution of organs equitably among transplant patients;
(E)[a] Adopt and use standards of quality for the acquisition and transportation of donated organs;
(F)[a] Prepare and distribute, on a regionalized basis (and, to the extent practicable, among regions or on a national basis), samples of blood sera from individuals who are included on the list and whose immune system makes it difficult for them to receive organs, in order to facilitate matching the compatibility of such individuals with organ donors;
(G)[a] Coordinate, as appropriate, the transportation of organs from organ procurement organizations to transplant centers;
(H)[a] Provide information to physicians and other health professionals regarding organ donation;
(I)[a] Collect, analyze, and publish data concerning organ donation and transplants;
(J) Carry out studies and demonstration projects for the purpose of improving procedures for organ procurement and allocation;
(K) Work actively to increase the supply of donated organs;
(L) Submit to the secretary an annual report containing information on the comparative costs and patient outcomes at each transplant center affiliated with the organ procurement and transplantation network;
(M) Recognize the differences in health and in organ transplantation issues between children and adults throughout the system and adopt criteria, polices, and procedures that address the unique healthcare needs of children;
(N) Carry out studies and demonstration projects for the purpose of improving procedures for organ donation procurement and allocation, including but not limited to projects to examine and attempt to increase transplantation among populations with special needs, including children and individuals who are members of racial or ethnic minority groups and among populations with limited access to transportation; and
(O) Provide that for purposes of this paragraph, the term "children" refers to individuals who are under the age of 18.

[a] Original 1984 language

medical judgement, prioritizing allocation based on urgency, ensuring that neither place of residence nor place of listing is a major determinant of access to a transplant, and reducing inter-transplant program waiting time variance with priority given to the most medically urgent status categories while avoiding futile transplants and organ wastage. The Final Rule also requires the OPTN to provide opportunity for its membership and other interested parties to comment on proposed policies. The OPTN is directed to take into account the comments received in developing and adopting policies. Finally, the Final Rule outlines provisions for anyone objecting to OPTN policies to appeal to the HHS secretary for review and adjudication.

With this regulatory framework in mind, and with input from HRSA representatives who serve as ex officio members of the OPTN/UNOS Board of Directors and committees, a framework for policy development was established (Fig. 1).

The Typical Policy Development Process

Identification of a Problem

The initial step in policy development is identification of an area where a new allocation policy is needed or an existing allocation policy needs improvement. Recognition of such "problems" can come from many different areas. Typically, new policy proposals arise as a part of the ongoing process of evaluation of OPTN performance with respect to its strategic priorities and alignment with the goals outlined in the Final Rule. However, OPTN committees can evaluate the need for new policy proposal in response to queries from external stakeholders (i.e., professional societies), OPTN members, or individuals.

The next step involves developing a project plan. The responsible OPTN Committee will develop a series of questions to define the evidence basis for the problem, develop data requests, sometimes including requests for data analysis from the Scientific Registry of Transplant Recipients (SRTR) to provide supporting evidence, consider the problem in the broader context of OPTN policy, assess the need for engagement with other OPTN committees and external stakeholders, and finally determine the steps needed (i.e., public fora) to establish consensus.

Proposed projects developed in this manner are reviewed by the OPTN/UNOS Policy Oversight Committee (POC) and prioritized with respect to the OPTN strategic plan. The OPTN/UNOS Executive Committee uses the POC prioritization to determine which projects will be granted financial, staff, and information technology (IT) resources necessary to proceed to the policy development phase.

Developing a Policy Proposal

The next step in the policy development process involves evaluation of the results of initial data requests in order to

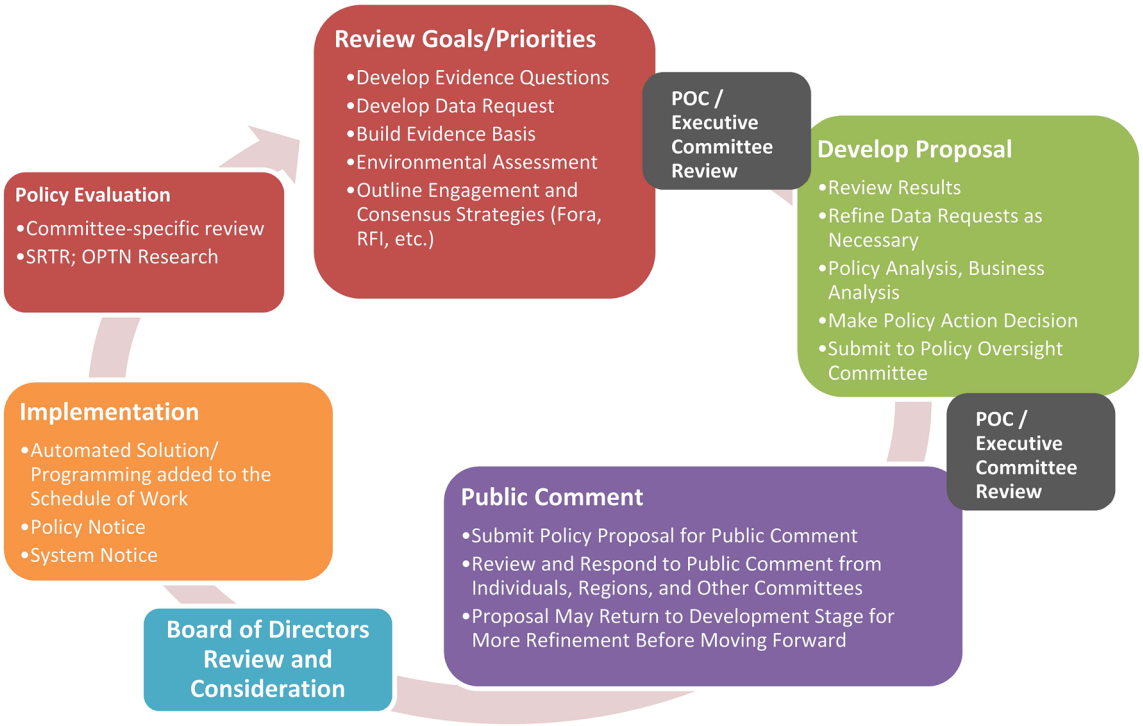

Fig. 1 OPTN framework for policy development

validate the evidence basis for the problem. With this in hand, the committee proceeds to consider how to address the identified problem. Options include not only changes to policy but also guidance to organ-specific review boards and educational initiatives directed at member transplant programs, organ procurement organizations (OPOs), or histocompatibility laboratories. In considering allocation policy changes, the committee may also seek to model the impact of proposed changes using simulated allocation models (SAM) provided by the SRTR. In addition to evaluating the impact of proposed policy changes on the problem at hand, an analysis of the resource implications for the OPTN and its members is also made. These steps are often an iterative process which, for some complex policy proposals such as the recent Kidney Allocation System revision, can take months to years and may sometimes include public fora intended to gather specific feedback in advance of developing a final proposal.

Once the Committee has determined the best course of action to address the problem, a PC proposal is prepared for submission in one of the biannual PC cycles. The proposal is then reviewed by the OPTN/UNOS POC and the executive committee. Both consider the document for completeness (including evidence that engagement with the appropriate OPTN committees and external stakeholders has occurred) and alignment with the Final Rule and the OPTN strategic plan. The Executive Committee also considers the proposal in the context of OPTN resources (both financial and IT capacity to implement the proposal).

Public Comment

Approved policy proposals are then distributed for PC. The OPTN holds two PC cycles each year, one in the winter and one in the fall, each are required to be at least 45 days in duration. Public comment documents are published on the OPTN website (http://optn.transplant.hrsa.gov/governance/public-comment/) providing the opportunity for anyone to enter a comment as well as view previously posted comments. External stakeholder organizations typically submit PCs through this portal. Feedback from each of the 11 OPTN regions sought during biannual regional meetings held during the PC period and feedback from each of the OPTN/UNOS committees are also posted to the OPTN website. A typical OPTN PC cycle involves between 10 and 20 policy proposals.

Following closure of the PC cycle, the Committee reviews the comments received, collaborates with relevant OPTN committees and external stakeholders, and develops responses to the major themes in public comment feedback. Based on this feedback, the Committee can decide to submit the proposal to the OPTN/UNOS Board for final approval as written or with revisions responsive to the PC, return the proposal to the development stage for further refinement, or discontinue work on the proposal. For policies to be brought forward to the OPTN Board, a briefing paper is prepared which includes the final proposed policy language and summarizes the response of the Committee to the received PC.

OPTN Board Review and Approval

Proposals submitted to the OPTN Board for approval are presented by the committee chair and discussed at one of the biannual OPTN board meetings. Board members review the provided briefing paper in advance of the meeting and consider whether to submit amendments for consideration in advance of the discussion. The OPTN Board may pass the proposal as written, pass an amended version, return the proposal to the committee for further refinement, or take another action. The OPTN/UNOS Executive Committee may also consider policy proposals and take action between meetings of the board of directors as required in special circumstances.

Once a policy proposal is approved, OPTN members are provided notification of the planned policy change, including an implementation date (which may be dependent on available IT resources if modifications to OPTN computer systems are required) and any required transition steps.

Post-implementation Monitoring

All policy proposals include a description of plans to monitor the proposal to ensure that it achieves its intended goal. Monitoring can include planned review of data by the sponsoring committee and/or other committees using data provided by OPTN and/or SRTR analysts. If areas where policies fail to achieve intended goals are identified, the committee will consider beginning a new project with the goal of addressing these gaps.

As described above, policy development is an iterative, evidence-based, consensus-driven process involving input from OPTN committees and regions, stakeholder organizations, and the general public. HRSA oversight exists throughout the process. Prior to modifications to the policy cycle dates in 2014, this process took a minimum of 18–24 months from concept to implementation.

Alternative Paths for Policy Development

Occasionally, circumstances arise where policy development cannot take the deliberate path outlined above. Examples of such instances include development of new technology, adoption of new regulations, and judicial intervention.

New Technology

Development of a total artificial heart (TAH) device capable of being managed as an outpatient created a situation where an existing policy created a potential disadvantage for patients.

In May, 2010, the OPTN became aware of two hospitals participating in an investigational device exemption (IDE) study to assess the effectiveness of the SynCardia Freedom™ Driver system which proposed to include as many as 60 patients discharged with the portable driver.

For heart transplant candidates with a TAH to qualify for the highest urgency listing status (1A), the heart allocation policy at that time required candidates to be admitted to the listing hospital. This status was granted for 14 days and could be renewed in 14-day increments indefinitely. Candidates not admitted to the listing hospital would be listed at the second status tier (1B) unless they had suffered a device complication. In contrast, patients receiving ventricular assist device (VAD) support could be listed at status 1A for up to 30 days, regardless of hospitalization status. VAD patients can also qualify for status 1A in the event of a device complication. In the absence of data to suggest that patients with a TAH would have decreased medical urgency for transplant upon discharge from the hospital, the thoracic committee concluded that forcing patients participating in the IDE study to downgrade to status 1B would medically disadvantage a population of patients contributing to medical innovation.

In order to address concerns raised by the community, the thoracic committee proposed a compromise amendment to the heart allocation policy to allow outpatient TAH candidates to be eligible for listing as status 1A for 30 days after discharge [3]. Because this situation placed heart transplant candidates at risk, the OPTN Board approved the policy amendment to be implemented concurrent with PC with a 1-year sunset date. Thus, the OPTN was able to make a change in policy in response to an unanticipated technology development that placed candidates at risk more rapidly than would have occurred with the standard policy development cycle

New Regulations

The addition of vascularized composite allograft (VCA) transplantation to the scope of oversight of the OPTN required action outside the standard policy development process because the federal regulation became effective before new VCA membership and allocation rules could be approved and implemented using the more deliberate process.

VCA refers to transplants composed of several different kinds of tissues (i.e., skin, muscle, bone), such as those in the hand, arm, or face, transferred from donor to recipient as a single functional unit. As this field matured, in March, 2008, the US Health and Human Services Department (HHS) began the process of establishing oversight of VCA, publishing a request for information (RFI) seeking input on whether VCAs should be included within the OPTN Final Rule's definition of organs. The RFI also sought input on whether VCAs should be added to the definition of human organs covered by section 301 of NOTA. Based on this RFI, in December 2011, HHS published a notice of proposed rulemaking to include VCA within the definition of organs covered by the OPTN Final Rule and NOTA. In July, 2013 HHS directed the OPTN

to establish policies regarding VCA transplantation with the goal of instituting a basic framework for VCA transplantation prior to implementation of the Final Rule modifications in July 2014. An OPTN VCA committee was established in order to accomplish this goal and presented a proposal to the OPTN Board for its June 2014 meeting. Because of the pending statutory change at the time, these policy changes were approved by the OPTN Board at that time with a "sunset" date of September 1, 2015 and a concurrent PC proposal was submitted [4].

Thus, the OPTN was able to make a policy change outside the standard PC process in response to constraints imposed by HHS regulatory authority.

Judicial Intervention

The need for organ allocation policies is driven by the fact that the demand for deceased donor organs in the USA far exceeds the current supply, resulting in more than 10,000 patients each year who are listed for transplant but die prior to receiving an organ. Although allocation policies attempt to balance justice and utility and have been developed by the OPTN through an evidence-based, consensus-driven process, inevitably circumstances will arise where patients and/or physicians assert that the system does not allocate organs fairly. Indeed, existing allocation policies acknowledge this reality by providing for "review boards" which adjudicate requests for adjustments to allocation priority based on the unique clinical circumstances of the patient as articulated by their transplant center. Such review boards exist within the allocation policies for each of the "life-saving" organs (i.e., liver, heart, lung).

A gap in the policies for such a review was exposed in June 2013 when a legal challenge was filed regarding the OPTN's lung allocation policy. The legal challenge asserted that a pediatric lung transplant candidate under the age of 12 was disadvantaged because donor lungs are allocated differently to children in this age range. Although adolescents and adults are prioritized using a lung allocation score (LAS), a model which ranks patients based primarily on predicted survival benefit, children under 12 are ranked by priority and waiting time. The developers of the LAS opted for this approach because there are too few lung transplants in the younger age group to provide sufficient numbers for modeling this patient population [5]. This distinction is balanced by ensuring that children under 12 receive priority for organs from donors under 12—the group most likely to provide suitably sized organs. In May 2013, a transplant center with a pediatric lung candidate under the age of 12 sought an appeal to allow the patient to be listed as an adolescent, be given an LAS score, and thus receive priority in the same category as adolescent and adult lung transplant candidates. Unfortunately, the OPTN lung allocation policy did not provide a mechanism for such an appeal to be considered by the lung review board.

As a result of the legal challenge, a federal judge ordered Secretary Sebelius to direct the OPTN to "immediately cease application of the Under 12 Rule as to the patient" [6, 7••].

After complying with the judge's order, the OPTN/UNOS Executive Committee held an emergency meeting to review recommendations from the thoracic, pediatric, and ethics committees regarding the pediatric lung allocation policy. At this meeting, the Executive Committee approved an amendment to the lung allocation policy which provides the lung review board with an exception pathway that allows pediatric lung transplant candidates aged 0–11 to be listed as adolescents [8]. Consistent with prior actions taken by the Board without PC, this amendment included a sunset date of June 2014 and was released for PC in the period that followed the Executive Committee's action [9].

Recent Changes to the Policy Development Process

The 2012–2015 OPTN strategic plan had a stated objective of "improving the policy development process to be more responsive to OPTN members." The above examples highlighted the need for the OPTN Board and Executive Committee to have flexibility in addressing urgent issues before a full public comment period could be completed. While the OPTN/UNOS Board and Executive Committee had the authority to take these actions, the process was not outlined in the bylaws in a transparent way. Accordingly, the OPTN Executive Committee developed a working group to review the policy development process in 2014. As a result of the recommendations of this working group, the OPTN Board approved a bylaws change [10•] which established formal requirements for an emergency policy development process. Emergency actions taken by the Board:

- Must be in response to a statutory or regulatory change, an emergent public health *or* patient safety issue or necessitated by a new medical device *or* technology that affects organ allocation
- Must have a sunset date no greater than 1 year from the policy's effective date
- Must be distributed for a PC period no more than 6 months from the policy's effective date

The Board also approved a bylaws change [10•] allowing for an expedited policy approval pathway for policy changes expected to be non-controversial (such as updates to the coefficients used to calculate scores used in allocation algorithms). Expedited actions must

- Be specified as policy language in a new or revised policy stipulating components of the policy eligible for future expedited updates.

- Subsequently be submitted for a PC period of no less than 30 days
- Must go through the normal PC process if objections are received from five members of the public, another OPTN committee, or four members of the Board of Directors
- May be submitted to the OPTN Board for approval if objections received do not meet the above threshold.
- Will otherwise become effective automatically upon notice to the OPTN membership.

The Executive Committee also reviewed the policy development timeline, noting that the time required from policy proposal concept inception to OPTN Board approval had increased from 90–104 during 2001–2005 to 243–291 days. When the time required for the programming changes to the OPTN computer systems is added to the development time, the total time often exceeded 2 years. A significant component of this increased time was due to misalignment of the PC cycles with the board meetings: there was not enough time between closure of the PC period and the next board meeting to allow proposals not requiring significant changes to be brought to the board (leading to a 5- to 6-month delay to the next board meeting). Therefore, the Committee recommended shortening the PC duration to 60 days and scheduling PC and OPTN Board meeting dates to allow sufficient time following PC closure for proposals to be considered at the board meeting immediately following the PC period. This timeline was implemented in 2015.

Future Challenges

An ongoing issue faced by the OPTN relates to alignment of OPTN policies with Medicare conditions of participation (COP) for organ procurement organizations and transplant programs published by the Centers for Medicare and Medicaid Services (CMS). For example, in order to preclude OPOs having to provide different versions of the same metrics to the OPTN and CMS, implementation of a policy passed by the OPTN/UNOS Board in June 2013, which updates the definitions of imminent and eligible deaths has been delayed because CMS has not yet updated the COP to reflect the new definitions [11]. Similarly, the board recently passed a policy proposal allowing OPOs to discontinue shipping paper copies of donor records with organs [12]. OPOs will not be able to take full advantage of this new policy unless CMS similarly changes the COP. Finally, the OPTN is currently exploring options to revise the metrics and mechanisms by which it evaluates transplant programs in an effort to reduce organ discards and increase transplants. Because CMS published evaluation criteria in 2007 [13] (which were well aligned with OPTN policy at the time) in order for any OPTN changes to have a meaningful impact on transplant center behavior, a mechanism to allow more facile coordination of OPTN and CMS policy changes will need to be developed. Otherwise, this OPTN effort, intended to reduce waiting list mortality and support innovation, will be limited.

Conclusion

In summary, in response to legislative (NOTA) and regulatory guidance (the Final Rule), the OPTN has developed an iterative, evidence-based, consensus-driven policy development process involving input from OPTN committees and regions, the donation and transplant community, and the general public. Recent refinements have stemmed from the recognized need for policy development to address situations requiring urgent action and develop efficient pathways for all instances. Opportunities remain, particularly with regard to alignment of OPTN policies with those developed by CMS.

Compliance with Ethical Standards

Conflict of Interest Stuart C. Sweet reports that he is currently serving as vice-president of UNOS. He was previously the secretary of UNOS and during that term served as the chair of the committee that recommended the improvements to the policy development process described in the article. My travel expenses related to these roles were paid by UNOS.

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Human and Animal Rights and Informed Consent This article does not contain any studies with human or animal subjects performed by any of the authors.

References

Papers of particular interest, published recently, have been highlighted as:
• Of importance
••Of major importance

1. National organ transplant act Pub. L. No. 98–507, Stat. 42 USC Sec. 274 (1984).
2. Organ procurement and transplantation network final rule, 42 CFR 121 (1998).
3. Proposal to allow outpatient adult heart transplant candidates implanted with total artificial hearts (TAH) thirty days of status 1A

time. 2011. http://optn.transplant.hrsa.gov/PublicComment/pubcommentPropSub_278.pdf. Accessed 11/23/2015 2015.
4. Implement the OPTN's oversight of vascularized composite allografts (VCAs). 2014. http://optn.transplant.hrsa.gov/media/1118/05_vca_implementation.pdf. Accessed 11/23/2015 2015.
5. Egan TM, Murray S, Bustami RT, Shearon TH, McCullough KP, Edwards LB, et al. Development of the new lung allocation system in the United States. Am J Transplant. 2006;6(5 Pt 2):1212–27.
6. Janet and Francis Murnaghan v. United States Department of Health and Human Services. Civil Action 13–3083. 2013. https://www.paed.uscourts.gov/documents/opinions/13D0477P.pdf. Accessed 11/23/2015 2015.
7.•• de Sante J, Caplan A, Hippen B, Testa G, Lantos JD. Was Sarah Murnaghan treated justly? Pediatrics. 2014;134(1):155–62. doi:10.1542/peds.2013-4189. **This is a thorough discussion of the ethical aspects of the case where policy change was driven by judicial intervention**.
8. Executive committee meeting June 10, 2013: agenda and materials. 2013. http://optn.transplant.hrsa.gov/ContentDocuments/OPTN_Exec_Comm_mtng_materials_06-10-13.pdf.
9. Proposal for adolescent classification exception for pediatric lung candidates. 2014. http://optn.transplant.hrsa.gov/PublicComment/pubcommentPropSub_345.pdf. Accessed 11/23/2015 2015.
10.• Improving the OPTN policy development process. 2015. http://optn.transplant.hrsa.gov/media/1120/07_executive_improving_policy_development.pdf. Accessed 11/23/2015 2015. **This OPTN policy proposal provides additional background and detail regarding recent improvements to the OPTN policy development process**.
11. Proposal to modify the imminent and eligible (I & E) neurological death data reporting definitions. 2012. http://optn.transplant.hrsa.gov/PublicComment/pubcommentPropSub_316.pdf. Accessed 12/4/2015 2015.
12. Reduce documentation shipped with organs. 2015. http://optn.transplant.hrsa.gov/media/1188/0815-08_Organ_Documentation.pdf. Accessed 12/4/2015 2015.
13. Hospital conditions of participation: requirements for approval and reapproval of transplant centers to perform organ transplants (CMS-3835-P), (2007).