# Report for the Percentage of Prevalent Patients Waitlisted (PPPW)

Submitted to CMS by the University of Michigan Kidney Epidemiology and Cost Center

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# Introduction

A measure focusing on the wait listing process is appropriate for improving access to kidney transplantation for several reasons. First, wait listing is a necessary step prior to potential receipt of a deceased donor kidney. Second, dialysis facilities exert substantial control over the process of waitlisting. This includes proper education of dialysis patients on the option for transplant, referral of appropriate patients to a transplant center for evaluation, assisting patients with completion of the transplant evaluation process, and optimizing the health and functional status of patients in order to increase their candidacy for transplant wait listing. These types of activities are included as part of the conditions for coverage for Medicare certification of ESRD dialysis facilities. In addition, dialysis facilities can also help maintain patients on the wait list through assistance with ongoing evaluation activities and by optimizing health and functional status. Finally, wide regional variations in wait listing rates highlight substantial room for improvement for this process measure [1,2,3].

This measure focuses specifically on the prevalent dialysis population, examining waitlisting status monthly for each patient. This allows evaluation and encouragement of ongoing waitlisting of patients beyond the first year of dialysis initiation who have not yet been listed. Patients may not be ready, either psychologically or due to their health status, to consider transplantation early after initiation of dialysis and many choose to undergo evaluation for transplantation only after years on dialysis. In addition, as this measure assesses monthly waitlisting status of patients, it also evaluates and encourages maintenance of patients on the waitlist. Maintenance of active status on the waitlist is important for increasing likelihood of transplantation [4] and thus by extension, is waitlisting overall. This is an important area to which dialysis facilities can contribute through ensuring patients remain healthy, and complete any ongoing testing activities required to remain on the wait list. In contrast to this measure, another waitlisting measure, the Standardized First Kidney Transplant Waitlist Ratio for Incident Dialysis Patients (SWR), focuses solely on new listing or living kidney donor transplantation within the first year after initiation of dialysis with the rationale of encouraging early access to transplantation or the wait list.

# Methods

## Overview

The Centers for Medicare & Medicaid Services (CMS) has contracted with the University of Michigan's Kidney Epidemiology and Cost Center (UM-KECC) to develop access to kidney transplantation measures for ESRD patients, including Standardized First Kidney Transplant Waitlist Ratio for Incident Dialysis Patients (SWR) for new patients and the Percentage of Prevalent Patients Waitlisted (PPPW) for the prevalent population.

The PPPW measure tracks the percentage of patients at each dialysis facility who were on the kidney or kidney-pancreas transplant waiting list. Results are averaged across patients prevalent on the last day of each month during the reporting year, adjusted for age.

## Data Sources

CROWNWeb (including CMS Medical Evidence Form (Form CMS-2728)) is the primary data source used for placing patients at dialysis facilities, age and incident comorbidities adjustments and exclusion of patients => 75 year-old (see information provided under "denominator details"). Organ Procurement and Transplant Network (OPTN) is the data source for waitlist or living donor transplant events. The

Nursing Home Minimum Dataset and the CMS Medical Evidence Form (Form CMS-2728) are used to identify SNF patients. A separate CMS file that contains final action claims submitted by Hospice providers was used to determine the hospice status.

## Outcome Definition

The numerator for the PPPW is number of patient months in which the patient at the dialysis facility is on the kidney or kidney-pancreas transplant waitlist as of the last day of each month during the reporting year.

## Denominator Definition

The denominator for the PPPW is the sum of all patient-months for patients who are under the age of 75 in the reporting month and who are assigned to the dialysis facility according to each patient's treatment history as of the last day of each month during the reporting year.

## Risk Adjustment

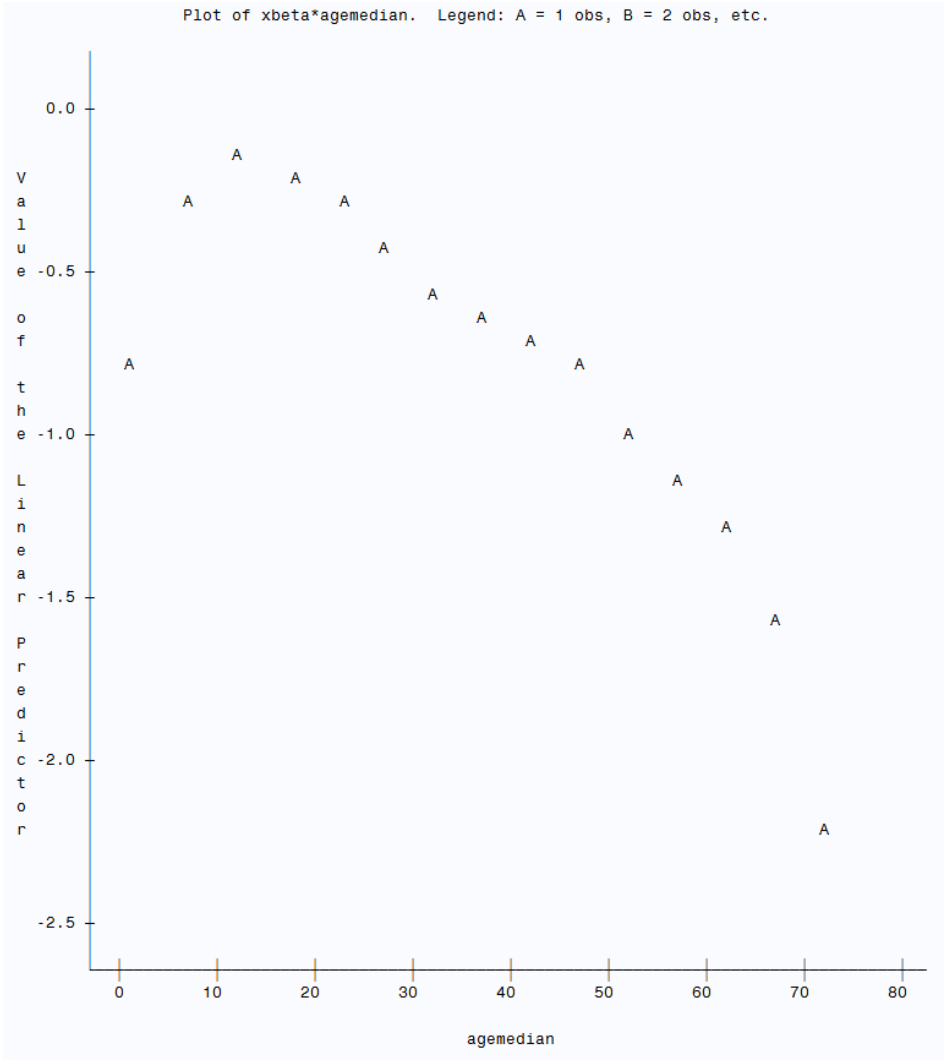
### Choosing Adjustment Factors

Age adjustment was deemed necessary on clinical grounds. Although age alone is not a contraindication to transplantation, older patients are likely to have more comorbidities and be generally more frail thus making them potentially less suitable candidates for transplantation and therefore some may be appropriately excluded from waitlisting for transplantation. This may affect waitlisting rates for facilities with a substantially older age composition than the average.

### Adjustment in PPPW

A linear spline was used to model the effect of (continuous) age. The spline's knots were determined empirically using standard techniques. Specifically, as an initial step, we categorized age into as many groups as the data would sustain (15 groups). We then estimated the effect of categorical age, then plotted the age-category-specific parameter estimates against their respective category-specific median ages. The shape of this plot indicates age intervals within which the slope is approximately constant, and similarly suggests ages at which the slope changes. Using this procedure and examining the plot in Figure 1, knots at 15, 55 and 70 were suggested.

**Figure 1. Plot of age trend (linear predictor versus median of age)**




## Exclusions

Exclusions that are implicit in the denominator definition include:

- Patients who were at age 75 or older in the reporting month.
- Patient who were admitted to a skilled nursing facility (SNF) or a hospice during the month of evaluation were excluded from that month; patients who were admitted to a skilled nursing facility (SNF) at incidence or previously according to Form CMS-2728 were also excluded.

The Nursing Home Minimum Dataset and the Questions 17u and 22 on CMS Medical Evidence Form are used to identify patients in skilled nursing facilities. For hospice patients, a separate CMS file that contains final action claims submitted by Hospice providers was used to determine the hospice status.

## Calculating PPPW

We assume a logistic regression model for the probability that a prevalent patient is wait-listed. Consider patient *i* at facility *j* during calendar month *k*; we set the response variate to $Y_{ijk}$ =1 if the patient is on the wait list and $Y_{ijk}$ 0 if not. The model is adjusted for age,

$$\text{logit}(p_{ijk}) = \alpha_j + \beta A_{ij},$$

coded as a linear spline with empirically determined knots at ages 15, 55 and 70. As such, the only factors in the logistic model are age and i and the facility indicators. The model is fitted using Generalized Estimating Equations (GEE; Liang and Zeger, 1986) in order to account for the correlation within-patient across months.

With over 6,000 facilities, it is difficult to estimate all parameters (i.e., including the facility indicators) simultaneously. Therefore, we break the fitting process into stages. At the first stage, we estimate the β vector by averaging 10 subgroups of approximately 600 facilities each. At the second stage, we then estimate the $\alpha_j$ ($j$=1, .., 6000) by fitting facility-specific intercept-only GEE models, with the linear predictor from the first stage, $\beta A_{ij,}$ serving as an offset. Per well-established GEE results (e.g., Liang and Zeger, 1986), the estimator of $\alpha_j$ is consistent for its target value, and follows a Normal distribution with standard error given by the robust 'sandwich' estimator computed via GEE. We can then compute $PPPW_j$ for each facility *j* as follows:

$$PPPW_j = \sum_i \sum_l \sum_k \; exp(\alpha_j + \beta A_{il}) \,/\, \{1 + exp(\alpha_j + \beta A_{il})\}. \,/\, n,$$

where *n* = total number of patient-months included in the overall study sample. The standard error of $PPPW_j$ is estimated through the Delta method; i.e., $SE(PPPW_j)=d_j \times SE(\alpha_j)$, where $d_j$ = $\sum_i \sum_l \sum_k \; exp(\alpha_j + \beta A_{il}) \,/\, \{1 + exp(\alpha_j + \beta A_{il})\}^2 \,/\, n$.

We then carry out a two-sided Wald test (0.05 significance level) that $PPPW_j=PPPW$, where *PPPW* equals the national average percentage waitlisted. Note that Wald the test is based on the logit of $PPPW_j$, which is much more likely to follow a Normal distribution than $PPPW_j$ itself, due to the symmetry and lack of range restrictions of the transformed version.

### Missing Data

Age is the only adjustment variable in the PPPW measure. Since age was calculated using the date of birth and the reporting month, and date of birth was required in our Standard Analysis Data Files, no missing value in age was identified in the patient population.

# Testing Results

Table 1. Coefficients and p-value in final PPPW model (note: $a_+=\max(a,0)$), 2016

| Covariate | Coefficient | p-value |
|---|---|---|
| **Age** | 0.06 | <.001 |
| **(age-15)$_+$** | -0.08 | <.001 |
| **(age-55)$_+$** | -0.03 | <.001 |
| **(age-70)$_+$** | -0.23 | <.001 |

The C-statistic (also known as the Index of Concordance) was 0.72. This indicates that the model correctly ordered 72% of the pairs of patient-months that were discordant with respect to the response variate. Month-specific C statistics were computed, in order to identify any trends by month in the model's discriminatory ability, and for computational ease.

## Reliability Testing

We used January 2016 – December 2016 data to calculate facility-level annual performance scores. The NQF-recommended approach for determining measure reliability is a one-way analysis of variance (ANOVA), in which the between-facility variation ($\sigma_b^2$) and the within-facility variation ($\sigma_{t,w}^2$) in the measure is determined. The inter-unit reliability (IUR) measures the proportion of the total variation of a measure (i.e., $\sigma_b^2 + \sigma_{t,w}^2$) that is attributable to the between-facility variation, the true signal reflecting the differences across facilities. We assessed reliability by calculating inter-unit reliability (IUR) for the annual performance scores. A small IUR (near 0) reveals that most of the variation of the measure between facilities is driven by random noise, indicating the measure would not be a good characterization of the differences among facilities, whereas a large IUR (near 1) indicates that most of the variation between facilities is due to the real difference between facilities.

Here we describe our approach to calculating IUR. Let $T_1,\ldots,T_N$ be the Percentage of Prevalent Patients Waitlisted (PPPW) for $N$ facilities. Since the variation in $T_1,\ldots,T_N$ is mainly driven by the estimates of facility-specific intercepts ($\alpha_1,\ldots, \alpha_N$), we use their asymptotic distributions to estimate the within-facility variation in PPPW. Applying the delta method, we estimate the variance of $T_i$ and denote the estimate as $S_i^2$. Calling on formulas from the one-way ANOVA, the within-facility variance in PPPW can be estimated by

$$s_{t,w}^2 = \frac{\sum_{i=1}^{N}\left[(n_i-1)S_i^2\right]}{\sum_{i=1}^{N}(n_i-1)},$$

and the total variation in PPPW can be estimated by

$$s_t^2 = \frac{1}{n'(N-1)}\sum_{i=1}^{N} n_i(T_i-\bar{T})^2,$$

where $n_i$ is the number of subjects in the $i$th facility, $\bar{T} = \Sigma\, n_i T_i / \Sigma\, n_i$, and

$$n' = \frac{1}{N-1}\left(\sum n_i - \sum n_i^2 \Big/ \sum n_i\right)$$

is approximately the average facility size (number of patients per facility). Thus, the IUR = $\sigma_b^2/(\sigma_b^2 + \sigma_{t,w}^2)$ can be estimated by $(s_t^2 - s_{t,w}^2)/s_t^2$.

The reliability of PPPW calculation only included facilities with at least 11 patients during the entire year.

The IUR value is 0.80. This value of IUR indicates that about four-fifths of the variation in the PPPW can be attributed to the between-facility differences (signal) and about one-fifth to within-facility variation (noise). This value of IUR implies a high degree of reliability.

## Validity Testing

### Systematic Assessment of Face Validity

The primary purpose of this measure is to increase access to kidney transplantation for patients on chronic dialysis. Because waitlisting is a crucial, necessary step prior to potential receipt of a deceased donor kidney, a measure which assesses waitlisting of patients by dialysis facilities has face validity as a measure of access to transplantation. Furthermore, a Technical Expert Panel (TEP), of 11 members consisting of transplant nephrologists, social workers, administrators and nurses with transplant process, policy and research expertise was convened. The TEP was charged with development of potential dialysis facility measures directed at improving access to transplantation. Although not unanimous, there was majority (by formal vote of 8-3) support for a dialysis facility measure related to waitlisting, on the basis that dialysis facilities importantly contribute to waitlisting of patients by helping them to navigate the process from referral through completion of the transplant evaluation, ensuring that all necessary testing as part of the evaluation process is done in a timely manner, and contributing to their overall health and therefore suitability for transplantation.

### Empirical Validity Testing

We assessed empirical validity of the measure by calculating Spearman correlations. Spearman correlation was selected because the data are rank-ordered (non-parametric data). Correlations were calculated to assess the association of the PPPW with other outcome quality measures. First, to demonstrate the relationship between PPPW and the anticipated outcome of increasing transplantation rates for patients at the facility, we examined the correlation of facility ranking with respect to the measure and the Standardized Transplant Ratio (STR, 2013-2016). The STR is the ratio of the actual number of first transplants to the expected number of first transplants for the facility, given the age composition of the facility's patients in 2013-2016. There are 4,857 facilities available for comparison. We expected to find that the PPPW and STR would be positively correlated.

We further examined the relationship between PPPW and a number of measures reflecting the quality of overall health care delivered to dialysis patients by facilities. These include the 2013-2016 Standardized Mortality Ratio (SMR), 2016 Standardized Hospitalization Ratio (SHR), 2016 Standardized Hospitalization Ratio (ED visits), and 2016 Standardized Readmission Ratio (SRR).

The Spearman correlation coefficient between facility waitlist rate and STR was significant: rho=0.45, p<.0001. There is also significant correlation between PPPW and the SMR (n=6,086, r=-0.11, p<.001), SHR (admissions) (n=6,400, r=-0.03, p<.001), SHR (ED visits) (n=6,400, r=-0.22, p<.001), and SRR (n=6,375, r=-0.03, p<.001). All results were as expected. Percentage of Prevalent Patients Waitlisted

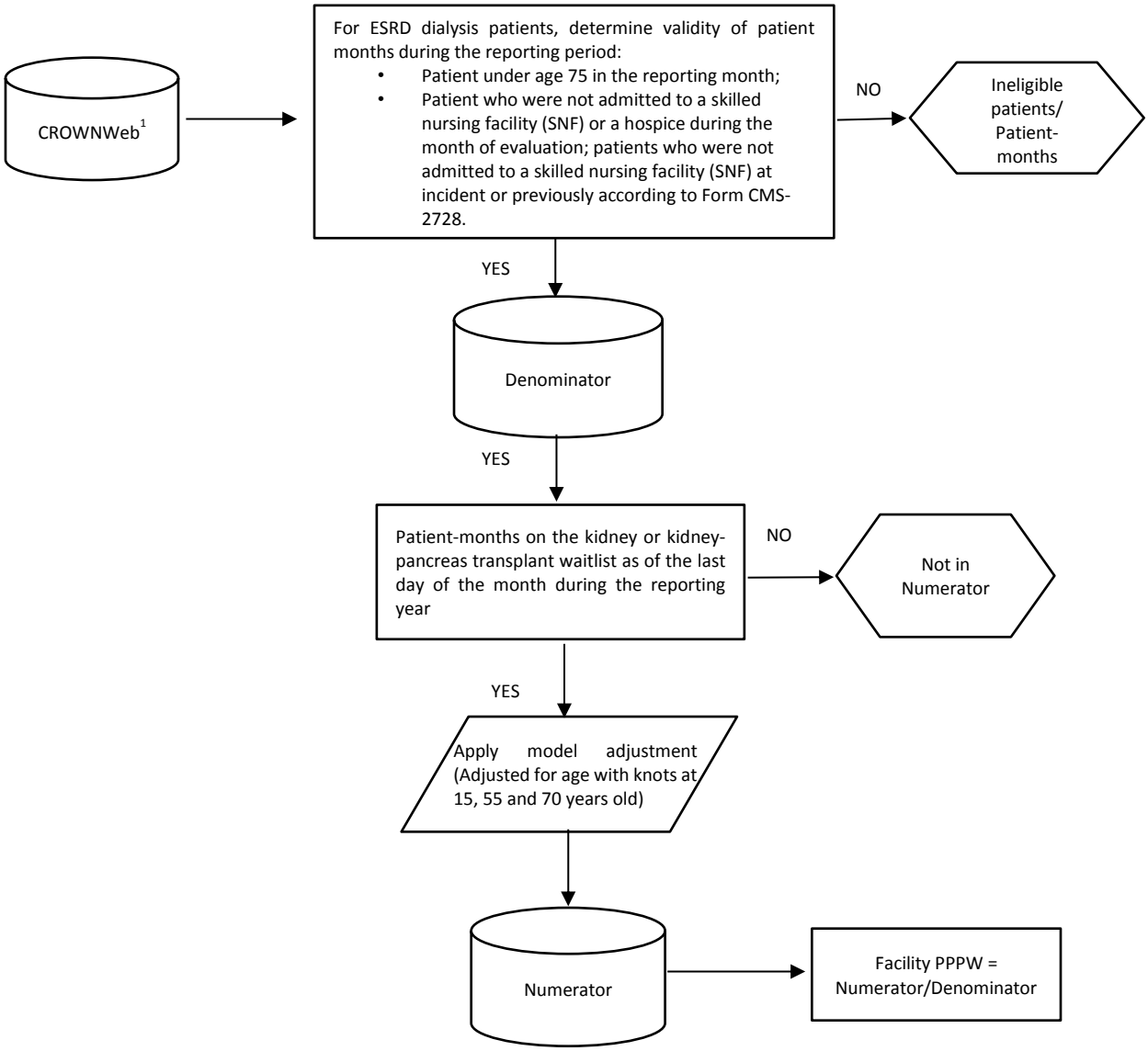
(PPPW) is positively correlated with STR, suggesting that facilities with higher waitlisting rates also have higher transplant rates. The Spearman correlation between PPPW and other measures indicates that higher waitlisted rate is associated with lower mortality rate, lower hospitalization rate and lower readmission rate.

## References

1. Ashby VB, Kalbfleisch JD, Wolfe RA, et al. Geographic variability in access to primary kidney transplantation in the United States, 1996-2005. American Journal of Transplantation 2007; 7 (5 Part 2):1412-1423.
2. Satayathum S, Pisoni RL, McCullough KP, et al. Kidney transplantation and wait-listing rates from the international Dialysis Outcomes and Practice Patterns Study (DOPPS). Kidney Intl 2005 Jul; 68 (1):330-337.
3. Patzer RE, Plantinga L, Krisher J, Pastan SO. Dialysis facility and network factors associated with low kidney transplantation rates among United States dialysis facilities. Am J Transplant. 2014 Jul; 14(7):1562-72.
4. Grams, M. E., Massie, A. B., Schold, J. D., Chen, B. P., & Segev, D. L. (2013). Trends in the inactive kidney transplant waitlist and implications for candidate survival. American Journal of Transplantation, 13(4), 1012-1018.

# Appendix

## Measure Calculation Flow Chart




[1] CROWNWeb is the primary basis for placing patients at dialysis facilities and dialysis claims are used as an additional source. Information regarding first ESRD service date, death, waitlist status and transplant is obtained from CROWNWeb (including the CMS Medical Evidence Form (Form CMS-2728) and the Death Notification Form (Form CMS-2746)) and Medicare claims, as well as the Organ Procurement and Transplant Network (OPTN) and the Social Security Death Master File. For denominator exclusions, the Nursing Home Minimum Dataset and the Questions 17u and 22 on CMS Medical Evidence Form are used to identify patients in skilled nursing facilities. Additionally, a separate CMS file that contains final action claims submitted by Hospice providers was used to determine the hospice status.

## Data Dictionary

| Variable | Primary Data Source |
|---|---|
| Facility CCN # | CMS data sources*1 |
| Reporting year and month | CROWNWeb |
| Waitlist status | Organ Procurement and Transplant Network (OPTN) |
| Date of Birth | CMS data sources*1 |
| Date of First ESRD | Medical Evidence Form (CMS-2728) |
| Nursing home status on the Medical Evidence Form *2 | Medical Evidence Form (CMS-2728) Question 17u and 22 |
| Nursing home status in the current month *2 | CMS Long Term Care Minimum Data Set (MDS) |
| Hospice status in the current month *2 | CMS Hospice file |

*1. CROWNWeb (including CMS Medical Evidence Form (Form CMS-2728)) is the primary data source used for placing patients at dialysis facilities, age and incident comorbidities adjustments and exclusion of patients ≥75 year-old . Organ Procurement and Transplant Network (OPTN) is the data source for waitlist or living donor transplant events. The Nursing Home Minimum Dataset and the CMS Medical Evidence Form (Form CMS-2728) are used to identify SNF patients. A separate CMS file that contains final action claims submitted by Hospice providers was used to determine the hospice status.
Unique patients are identified by using a combination of SSN, first name, surname, gender, Medicare claim number and birth date. A matching process is performed to ensure that minor typos and misspellings do not cause a patient record to fall out of their history. The matching process is able to successfully match 99.5% of patients. The remaining patients have incomplete or incorrect data that does not allow them to be matched.

*2. Exclusion factors