PREFERENCE-BASED ASSESSMENT


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ABSTRACT

Objectives: To measure the utility of patients with ureteral stones under various medical regimes and to identify significant factors affecting utility for various health states. Methods: A cross-sectional survey was conducted to measure the utility of 89 patients on each health state related to the clinical management of ureteral stones. Health states with respect to intervention and treatment modalities were classified into the acute phase (including medication, extracorporeal shock wave lithotripsy, ureterorenoscopic lithotripsy, and surgery) and the chronic phase (no specific intervention, lifestyle modification, maintenance with surveillance, and continued medication). Utility was measured by using the modified standard gamble. Demographic data and relevant history of treatment modalities and interventions for ureteral stones were collected by using a questionnaire. Results: Utility scores of health states in the acute phase (ranging from 0.914 [surgery] to 0.967 [extracorporeal shock wave lithotripsy]) were lower than those in the chronic phase (ranging from 0.955 [maintenance with surveillance] to 0.974 [lifestyle modification]). Utility for surgery was lower than for nonsurgical methods. Utilities for the two lithotripsy modalities were close to that for medication. The utility figures for health states in the chronic phase were the highest for lifestyle modification, but the differences across health states were trivial. Sex, history of ureterorenoscopic lithotripsy, education level, and employment were significant covariates in the final multiple linear regression model. Conclusions: A modified standard gamble chained method was applied to measure the utility for health states in relation to the clinical management of ureteral stones. Patients preferred nonsurgical treatment over surgical treatment and hemodialysis regardless of health states. We also found that sex, a history of ureterorenoscopic lithotripsy, education level, and employment affected utility for health states related to clinical management. Our findings provide an insight into patient preference for the choice of treatment of ureteral stones. Keywords: extracorporeal shock wave lithotripsy, standard gamble approach, ureteral stones, ureterorenoscopic lithotripsy, utility.

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Introduction

Clinical management of ureteral stone, an illness with a high likelihood of recurrence [1], was dominated by surgery before the advent of extracorporeal shock wave lithotripsy (ESWL) and ureterorenoscopic lithotripsy (URSL), which have been adopted as primary treatment modalities for removing ureteral stones [2]. These two treatments together with the high recurrence rate of ureteral stones leave patients in a dilemma regarding whether to undergo these treatments, particularly for prophylactic purposes for treating small silent renal stones. Although patient utility plays a crucial role in selecting the treatment plan for ureteral stones, few studies have been conducted to address this issue. To the best of our knowledge, only one previous study measured patient utility in the treatment of upper urinary tract calculi [3]. However, several concerns have been raised in this study. The classification for health states corresponding to each utility measurement is too broad to reflect the complex of health states involved in state-of-the-art clinical scenario of treating ureteral stones. Factors affecting patient preference have not been fully investigated. Because the patient preference may vary with time, place, and ethnic group, it is worthwhile to measure patient preference over the choice of treatment modality with refined classifications to adapt the updated treatment modalities and preventive strategies of ureteral stone and identify its associated factors.

Among three common direct eliciting methods—standard gamble (SG), time trade-off method, and visual analogue scale—for measuring the utility [4–12], each has strength and weakness in the aspects of practicality, reliability, and validity [4–14]. We prefer choice-based techniques (SG and time trade-off) to choice-less methods such as visual analogue scale [8,15]. Of the two choice-based methods, although the impact of three bias (probability weighting, loss aversion, and scale comparability) on utility curva-

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The modified SG method

Step 1: Measuring the absolute utility for hemodialysis
Because hemodialysis was the most serious complication in patients with ureteral stones in this study and is common in Taiwan, we assume it is the worst state for the subject alive but better than death. We used the SG method to estimate the absolute utility for hemodialysis, defined as $U_i$ (Fig. 1), by using healthy and dead as reference states with two extreme states of utility as “1” and “0,” respectively. To measure utility for a chronic health state, say state $i$, a scenario was described as follows: “A male diagnosed with end-stage renal disease has been treated with conventional treatment-hemodialysis three times per week for a long time. A new treatment is available that may render him completely healthy with probability $p$, but he also runs the risk of dying if the treatment fails.” Whether he accepts a new treatment depends on his preference over the utility of undergoing this new treatment determined by the degree of chance that makes him consider the new treatment as indifferent to the conventional method, one accepting the new treatment with probability $p$ of attaining the best state (healthy) and with failure probability $(1 - p)$ of becoming worse, or rejecting the new treatment and receiving the conventional treatment given the current state $i$ being measured. We used an iterative bidding process to identify $p$, which is utility for hemodialysis according to the expected utility theory [17], which indicates that the utility for state $i$ for the above scenario is equivalent to

$$U_i = pU_b + (1 - p)U_w$$

where $U_b = 1$ and $U_w$ represent the utility values of the best (healthy = 1) and the worst states (dead = 0), respectively.

Step 2: Ranking the states in the same phase
In our case, healthy status was always regarded as the best reference state (score 1), whereas the worse state depended on each subject’s ranking sequence. We used the four states in the acute phase for ureteral stone management as an example (see Fig. 1).

Methods

Study subjects
A cross-sectional survey was conducted in 267 patients aged 20 to 65 years from the Department of Urology of Min-Sheng General Hospital, Taoyuan, Taiwan, who sought medical treatment for different types of ureteral stones between March and May 2007. Of the 267, 118 were outpatients who did not require further therapy, 96 were being treated with ESWL, and 53 were being treated with URSL. Fifty-two patients were randomly selected to represent 118 outpatients. All patients treated with ESWL or URSL were enrolled. Ninety-six were being treated with ESWL, and 53 were being treated with URSL. Fifty-two patients were randomly selected to represent 118 outpatients who did not require further therapy, and 53 were being treated with URSL. Those in the chronic phase were classified as no specific intervention, lifestyle modification, maintenance with surveillance, and continued medication.

Health state of patients with ureteral stones
Utility measured in our study was for patients undergoing different treatments for acute and chronic ureteral stones. Treatments in the acute phase were subdivided into four categories: medication, ESWL, URSL, and surgery. Those in the chronic phase were classified as no specific intervention, lifestyle modification, maintenance with surveillance, and continued medication.

![Fig. 1 – Flowchart for ranking and measuring the relative utility between reference states. ESWL, extracorporeal shock wave lithotripsy; URSL, ureterorenoscopic lithotripsy.](image-url)

![Fig. 2 – Flowchart of the standard gamble method for measuring utility.](image-url)
Four states—ESWL, medication, URSL, and surgery—in the acute phase were ranked by each subject.

Step 3: Measuring relative utility

Figure 2 shows the process used to obtain the indifferent point between the target state and the reference states, which was similar to the method used in the previous study [18]. For example, say we want to measure a man’s utility for surgery, which he ranks as fourth among the four states in the acute phase, as shown in Figure 1.

In addition to death status, which is assumed as the worst utility here, the relative utility value of rank 4 was measured between the worst state (hemodialysis) and the best state (healthy) by creating the following scenario: “Would you choose a surgery that could render you completely healthy with probability p, but lead to hemodialysis with probability (1 – p) if the surgery fails?” Following the conventional SG method, we estimated the probability of the indifferent point, that is, utility, between the two options by approaching it from both the bottom side where \( p = 0 \) and the top side where \( p = 100\% \) following the iterative bidding method using 2.5% as an incremental unit similar to the procedure used by Bosch and Hunink [18].

The same procedure was repeated to attach a probability of the indifferent point for estimating the relative utility forURSL. Similarly, we measured the utility for other states with ranks 1 to 3. This procedure was also applied to measure utilities for the treatment of the chronic state.

Step 4: Using the chained method to convert relative utility into absolute utility

The chained method [19–21] was further adopted to convert relative utility into absolute utility as follows:

\[
U_4 = U_5 + (1 - U_5) U_{4-5}
\]

\[
U_5 = U_4 + (1 - U_4) U_{3-4}
\]

\[
U_3 = U_5 + (1 - U_5) U_{3-5}
\]

\[
U_2 = U_3 + (1 - U_3) U_{2-3}
\]

\[
U_1 = U_2 + (1 - U_2) U_{1-2}
\]

Suppose his or her \( U_{1}, U_{2}, U_{4}, \) and \( U_{5} \) were 0.5, 0.9, and 0.775, respectively. We can convert the relative utility to absolute utility of URSL, denoted as \( U_{URSL} \), and the top side where

\[
U_{URSL} = U_5 + (1 - U_5) U_{URSL-100}
\]

\[
U_{URSL} = U_4 + (1 - U_4) U_{URSL-URSL}
\]

\[
U_{URSL} = U_3 + (1 - U_3) U_{URSL-URSL}
\]

\[
U_{URSL} = U_2 + (1 - U_2) U_{URSL-URSL}
\]

\[
U_{URSL} = U_1 + (1 - U_1) U_{URSL-URSL}
\]

\[
U_{URSL} = 0.5 + (1 - 0.5) \times 0.9 + [1 - (0.5 + (1 - 0.5) \times 0.9)] \times 0.775
\]

\[
= 0.98875
\]

Other values were also converted in a similar manner.

Statistical analysis

Because the majority of absolute utilities of ureteral stones for different treatment conditions exceeded 0.9, we converted the range of utilities by using complementary log-log transformation for the purpose of conducting regression analysis [22]. The use of such transformed utility \( U^* \) [e.g., \(-\ln(-\ln(U))\)], where \( U_i \) is the original absolute utility score of state \( i \) is pursuant to the principle of generalized linear model [23] when the expected value of outcome such as utility is close to 1 [22]. Then, a multiple linear regression model was applied to estimate the effects of relevant covariates on the transformed utility. We estimated the expected individual utility score \( E(U) \) as follows:

\[
E(U) = \exp(-\exp(-[\alpha + \Sigma CX]))
\]

where \( \alpha \) is the intercept, \( X \) is the vector of a set of covariates that were statistically significant in the multiple linear regression model, and \( C \) is the corresponding regression coefficient for \( X \). We selected the covariates included in the multiple regressions with the “enter” method. The entry for the model for the covariates was set at \( p \) value less than 0.1, and the statistically significant level was set at 0.05. Adjusted \( R^2 \) values were also reported to estimate the proportion of variance explained by the covariates considered in the model with adjustment for the number of parameters given degree of freedom. Statistical analysis was implemented by using SAS 9.1 (SAS Institute, Inc., Cary, NC).

Results

We completed the utility measurement based on 89 patients. The response rate was 44% (89 of 201). Information on the characteristics of 56% dropout subjects was not available, but we believe the reasons for dropout may not be related to our utility measurement because patients who sought medical care in the same hospital in Taiwan would have similar background such as socioeconomic status. The average interview time was around 20 minutes. The mean age was 43 ± 10.4 years. The proportion of men was threefold than that of women. Around 68% of the subjects had education level at senior high school or above (Table 1).

Table 2 shows the mean value and SD for the patients with ureteral stones. Utility for hemodialysis was 0.399 ± 0.343. Utility for surgery was the lowest one after hemodialysis. Utility of health states in the acute phase was lower than those in the chronic phase. Utilities in the acute phase ranged from 0.914 (surgery) to 0.967 (ESWL). Those in the chronic phase ranged from 0.955 (maintenance with surveillance) to 0.974 (lifestyle modification). Utility for surgery was lower than those for nonsurgical methods. Utilities for the two lithotripsy modalities were close to that for medication. The utility figure for health states in the chronic phase was the highest for lifestyle.

<table>
<thead>
<tr>
<th>Variable</th>
<th>Mean ± SD or proportion</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age (y)</td>
<td>43 ± 10.4</td>
</tr>
<tr>
<td>Sex, n (%)</td>
<td></td>
</tr>
<tr>
<td>Man</td>
<td>67 (75)</td>
</tr>
<tr>
<td>Woman</td>
<td>22 (25)</td>
</tr>
<tr>
<td>Education level, n (%)</td>
<td></td>
</tr>
<tr>
<td>Primary school</td>
<td>14 (16)</td>
</tr>
<tr>
<td>Junior high school</td>
<td>14 (16)</td>
</tr>
<tr>
<td>Senior high school</td>
<td>28 (31)</td>
</tr>
<tr>
<td>Undergraduate</td>
<td>30 (34)</td>
</tr>
<tr>
<td>Graduate</td>
<td>3 (3)</td>
</tr>
<tr>
<td>Working status, n (%)</td>
<td></td>
</tr>
<tr>
<td>None</td>
<td>17 (19)</td>
</tr>
<tr>
<td>Part-time</td>
<td>5 (6)</td>
</tr>
<tr>
<td>Full-time</td>
<td>67 (75)</td>
</tr>
<tr>
<td>History of ESWL, n (%)</td>
<td></td>
</tr>
<tr>
<td>No</td>
<td>13 (15)</td>
</tr>
<tr>
<td>Yes</td>
<td>76 (85)</td>
</tr>
<tr>
<td>History of URSL, n (%)</td>
<td></td>
</tr>
<tr>
<td>No</td>
<td>29 (33)</td>
</tr>
<tr>
<td>Yes</td>
<td>50 (56)</td>
</tr>
</tbody>
</table>

ESWL, extracorporeal shock wave lithotripsy; URSL, ureterorenoscopic lithotripsy.
modification, but the differences across health states were trivial.

Table 3 shows the results analyzed with the multiple linear regression model by each state. Sex and history of URSL were two major significant covariates in the final model, whereas education and employment may also make contributions but depend on the health states of ureteral stone management. Note that age, history of ESWL, and history of surgery were not statistically associated with any health state. None of the covariates was statistically significant in the regression analysis for no specific intervention (data not shown). Regardless of health states, utility of women was consistently lower than that of men. Those who had previous URSL had higher utility than those who had not. The significant associations were found not only for health states in the acute phase but also for those in the chronic phase. Those with a lower education level had lower utility than did those with a high education level, but the statistically significant associations were noted only for the two health states of medication and URSL in the acute phase. Those who were employed had a higher utility than those who were unemployed, but the significant association was noted only for medication in the acute phase. The bottom panel of Table 3 shows the results of adjusted $R^2$. The values in the acute phase were higher than those in the chronic phase. The highest was URSL (14.24%) followed by medication (13.68%) and ESWL (10.33%). The adjusted $R^2$ values were lower than 10% for surgery and other health states in the chronic phase (Table 3).

Table 4 shows the results of regression analysis by combining all nonsurgical methods as one group in the acute phase. The utility for surgery was statistically significantly lower than that for nonsurgical treatment in the acute phase after adjusting for other covariates. Again, only significant covariates (including sex, previous URSL, education level, and employment) identified in univariate analysis were included in the final model.

Discussion

Because patient preference plays a crucial role in selecting the treatment plan for the clinical management of ureteral stones, this study used a modified SG method following the axioms of Neumann–Morgenstern utility theory [17], where all treatments during each phase in this study were ranked by patients and the indifferent point between the two anchors was identified to measure the utility for different treatments and interventions. We also identified demographic features and relevant factors responsible for utility.

In contrast to the previous study [3] addressing the measurement of patient utility for choosing different treatment modalities, the differences across health states were trivial.

Table 2 – Distribution of the utility score (n = 89).

<table>
<thead>
<tr>
<th>Phase and state</th>
<th>Mean ± SD</th>
</tr>
</thead>
<tbody>
<tr>
<td>Treatment in the acute phase</td>
<td></td>
</tr>
<tr>
<td>Medication</td>
<td>0.963 ± 0.108</td>
</tr>
<tr>
<td>ESWL</td>
<td>0.967 ± 0.107</td>
</tr>
<tr>
<td>URSL</td>
<td>0.953 ± 0.124</td>
</tr>
<tr>
<td>Surgery</td>
<td>0.914 ± 0.159</td>
</tr>
<tr>
<td>Intervention in the chronic phase</td>
<td></td>
</tr>
<tr>
<td>No specific intervention</td>
<td>0.959 ± 0.090</td>
</tr>
<tr>
<td>Lifestyle modification</td>
<td>0.974 ± 0.076</td>
</tr>
<tr>
<td>Maintenance with surveillance</td>
<td>0.971 ± 0.079</td>
</tr>
<tr>
<td>Continued medication</td>
<td>0.955 ± 0.088</td>
</tr>
<tr>
<td>Hemodialysis</td>
<td>0.399 ± 0.343</td>
</tr>
</tbody>
</table>

ESWL, extracorporeal shock wave lithotripsy; URSL, ureterorenoscopic lithotripsy.

Table 3 – The multiple linear regression analysis for the transformed utility values for ureteral stone measurement for different states.

<table>
<thead>
<tr>
<th>Variable</th>
<th>Acute phase</th>
<th>Chronic phase</th>
<th>Hemodialysis</th>
</tr>
</thead>
<tbody>
<tr>
<td>Intercept</td>
<td>10.995 (1.725)</td>
<td>7.459 (0.504)</td>
<td>6.449 (0.109)</td>
</tr>
<tr>
<td>Sex (woman vs. man)</td>
<td>1.567 (0.879)</td>
<td>2.153 (0.868)</td>
<td>1.459 (0.639)</td>
</tr>
<tr>
<td>URSL experience (yes vs. no)</td>
<td>1.567 (0.879)</td>
<td>2.153 (0.868)</td>
<td>1.459 (0.639)</td>
</tr>
<tr>
<td>Education (senior high school and above vs. under)</td>
<td>0.854 (0.384)</td>
<td>0.512 (0.282)</td>
<td>0.839 (0.370)</td>
</tr>
<tr>
<td>Employment (employed vs. unemployed)</td>
<td>0.854 (0.384)</td>
<td>0.512 (0.282)</td>
<td>0.839 (0.370)</td>
</tr>
<tr>
<td><strong>Adjusted $R^2</strong></td>
<td>0.1424</td>
<td>0.1033</td>
<td>0.0449</td>
</tr>
</tbody>
</table>

Note. The variables included in the final model at a significance level of $P < 0.1$.
Table 4 – The difference of utility score between surgical and nonsurgical in the acute phase after adjusting for other covariates.

<table>
<thead>
<tr>
<th>Variable</th>
<th>Estimate</th>
<th>SD</th>
<th>P</th>
</tr>
</thead>
<tbody>
<tr>
<td>Intercept</td>
<td>8.157</td>
<td>0.879</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Surgical vs. nonsurgical</td>
<td>-3.187</td>
<td>0.389</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Sex (woman vs. man)</td>
<td>-2.204</td>
<td>0.411</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Education (senior high school and above vs. under)</td>
<td>-0.502</td>
<td>0.163</td>
<td>0.0022</td>
</tr>
<tr>
<td>Employment (employed vs. unemployed)</td>
<td>2.252</td>
<td>0.737</td>
<td>0.0024</td>
</tr>
<tr>
<td>ESWL experience (yes vs. no)</td>
<td>1.029</td>
<td>0.486</td>
<td>0.0348</td>
</tr>
<tr>
<td>URSL experience (yes vs. no)</td>
<td>1.347</td>
<td>0.369</td>
<td>0.0003</td>
</tr>
<tr>
<td>Adjusted $R^2 = 0.258$.</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

ESWL, extracorporeal shock wave lithotripsy; URSL, ureterorenoscopic lithotripsy.

... for ureteral stones, the features in our study distinct from their study are several-fold. First, the worst group except death used in their study was based on patients with nephrectomy, whereas the corresponding group used in our study was based on patients with hemodialysis because nephrectomy is rare in Taiwan. Second, health states considered in their study for measuring utility were different from ours. We separated treatment into acute and chronic phases. For example, the acute phase in our study was given a new state. As far as the chronic phase is concerned, they considered only medication but we took into account other methods such as lifestyle modification, no specific intervention, and surveillance. Third, we used the chained method to convert relative utility into absolute utility. The reason is that our classification of health states included temporary and nonrisky health states, although our chained method is more complicated than the previously used chained method [19–21].

Compared with the previous study [3], utility for surgery was lower than that for nonsurgical method as seen in both studies. By looking at absolute figures with similar health states, utility for surgery in their study was 0.93 (0.01–0.99), which was slightly higher than that obtained in our study, 0.91 (0.24–0.99). Utility for long-term medication in their study was slightly lower than that in our study (0.94 ± 0.15 vs. 0.96 ± 0.088). However, the SD of ours is smaller than theirs.

Because patient utility varies from individual to individual, identifying factors affecting patient utility for health states related to the choice of treatments is worthy of being investigated and has been barely addressed. Factors affecting utility identified in our study included sex, history of URSL, and employment. Those patients with history of URSL had higher utility in those conditions (including ESWL, URSL, lifestyle modification, maintenance with surveillance, continued medication) than did those without a history of URSL whereas it did not affect utility for surgery and the irreversible renal failure condition such as hemodialysis. These findings imply that patients after experiencing the effective URSL were optimistic toward all noninvasive treatment or preventive strategy. The reason of having lower utility for people with high education level in URSL and medication may be due to a higher expectation toward the effectiveness of two treatment modalities in relieving the pain caused by ureteral stones compared with those with low education level. The finding that employment was associated only with medication may be explained by the concern over time cost that encourages them to take medication rather than other time-consuming treatments. However, the true causes should be validated in the future. In addition, the regression coefficients of statistically significant items presented in Table 3 would give an individual patient expected utility using the equation. For example, the expected utility score of ESWL for a female with a previous URSL equals exp[-exp{-(7.450 – 0.207 × (female = 1) + 2.135 × (history of previous URSL = 1))}] = 0.9995 and that of continued medication equals exp[-exp{-(4.513 – 1.067 × (female) + 1.055 × (history of previous URSL))}] = 0.9890. Individuals with different combinations of covariates can be estimated in the same way. Predicating an individual utility with different characteristics may make a contribution to considering individual-tailored decision making on treatment modality and preventive strategies of ureteral stones in the future.

There are several concerns and limitations in our study. The first concern is that utility for treatment in the acute phase ranged from 0.914 (surgery) to 0.967 (ESWL). One reason for such a narrow range and higher utility may be that modified SG was used to measure preferences over quality of life resulting from treatment rather than treatment regimen. Consequently, although different treatments are given to patients, quality of life after treatment tends to be similar and utilities are not much different. However, from the methodological viewpoint, this may also be related to the application of the modified SG method that has been consistently reported to have an upward bias resulting from probability weighting [13,15,16]. The second limitation is a low response rate (44%). The major reason is that the interview with modified SG procedure is time-consuming, and participants may refuse to participate. The third concern is that as the adjusted $R^2$ is not very high, this suggests that utility may vary by other factors not measured in this study. Moreover, a lower adjusted $R^2$ in the chronic phase compared with that in the acute phase may suggest a further study to identify other factors accounting for the utility of health states in the chronic phase.

In conclusion, a modified SG chain method was applied to measure the utility of health states in relation to the management of ureteral stones. Patients tend to prefer nonsurgical treatment over surgical treatment and hemodialysis. We also found that sex, a history of URSL, education level, and employment affected utility. Our findings provide an insight into patient preference for the choice of treatment for ureteral stones. Source of financial support: The authors have no other financial relationships to disclose.

RECENTES


