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Results of a population survey

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Evidence of a public preference for funding high cost ultra rare disorders: Results of a population survey[1]

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Abstract

This paper suggests and tests a reason why the public might wish to allocate resources to 'cost ineffective' – effective but high cost – health services for a small number of patients when the resources might produce more health if they were allocated to cost effective services. As citizens, people are postulated to be less concerned with net benefits than with the fairness of the share of costs and benefits borne by the different individuals who experience them. With benefits and costs shared by a small and a large number of people respectively, average benefits to recipients will be high and average costs to payers will be low. When sharing reduces the cost to any individual sufficiently, citizens with communitarian values which include a 'duty of care', may prefer to share the small cost per person of very expensive and 'cost ineffective' services for the severely ill rather than maximise global net benefits.

Two surveys are reported in which respondents were asked to allocate a budget between cost effective treatments which have a small effect upon a large number of relatively well patients and high cost, 'cost ineffective' treatments which benefit a small number of severely ill patients. Results are consistent with the sharing hypothesis and indicate likely public support for the funding of some of the expanding number of high cost treatments for ultra rare disorders (URDs).

Keywords: ultra rare disorders (URDs); orphan drugs; CEA; equity efficiency trade-off; communitarianism; sharing; orphan drugs; rare diseases

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1 Introduction

The theory of economic evaluation commonly commences with the assumption that, all else equal, the social objective of the health sector is the maximisation of quality adjusted life years (QALYs). With a fixed budget, this is achieved by selecting services with the lowest cost per QALY. However it is widely recognised that the simple ‘QALY model’ may omit important considerations, with a recent review of the field concluding that ‘the recognition that CEA (cost effectiveness analyses) cannot readily accommodate every concern…has led nearly every group that recommends the use of CEA…or that uses it directly…also to recommend that decisions…should take into account important factors that are not embedded in the analysis’ (Garber and Sculpher, 2011 p 493). Similarly, in their text Drummond et al. (2005 p 47) note that ‘economic evaluation does not usually incorporate the importance of the distribution of costs and consequences…yet, in some cases, the identity of the recipient group…may be an important factor in assessing the social desirability of a service or program’.

In response to these concerns there has been a growing literature which focuses upon the variables omitted from the ‘QALY model’ which may be important for social welfare. The literature is primarily empirical and research has sought to elicit public preferences for the inclusion of the omitted variables. A number of reviews of this literature now exist, for example Nord (1999), Dolan Shaw et al (2005), Stafinski et al. (2011), Whitty et al (2014), Gu et al. (2015). Some variables are identified in all of these reviews as being potentially important. These include the severity of untreated illness and the age of the recipient. Studies concerning severity have been reviewed by Nord and Johansen (2014) and age related studies by Keeley et al. (2012). Other candidates for consideration are the realisation of a person’s health potential, the achievement of a lifetime ‘fair innings’, a patient’s social or economic contribution and the concentration or dispersion of the health benefits. A common feature of these suggested additions to the ‘QALY model’ is that they affect the benefit side of the cost per QALY ratio.

However a limited number of studies have observed a public preference for allocating some part of a limited budget to services that are efficacious but ‘cost ineffective’ because of their high opportunity cost (Nord et al., 1995, Abellan-Perpinan and Pinto-Prades, 1999, Ubel and Loewenstein, 1996, Ratcliffe, 2000, McKie et al., 2011, Richardson et al., 2012). In each of these studies survey respondents were asked to allocate resources to individuals who would benefit less than an alternative, clearly identified group or individual, despite the absence of characteristics of the benefits or the beneficiaries which appeared to justify this. In one of these studies, members of focus groups explained their budgetary allocation to cost ineffective services in terms of the preservation of hope and the unfairness of removing any possibility, that a patient might be the lucky recipient of the limited service (McKie et al., 2011). A similar explanation was suggested by Nord et al. (1995).

The present study tests an additional hypothesis which relates to cases where the high cost of a small number of effective services results in their cost ineffectiveness as currently assessed. The hypothesis is that the sharing of costs per se will have an independent effect upon the evaluation of the importance of cost.

Cost sharing occurs in any system of pooled funding and community insurance. But under the influence of economic theory there is usually an effort to limit sharing to the subset of services which are ‘cost effective’. The assessment of this is not affected by the fact that the cost of the service will subsequently be shared and the number of people sharing the cost is irrelevant: cost effectiveness is the same if costs fall upon a single person or are shared between many people. However the sharing of costs may affect their actual and perceived importance for wellbeing. First, as the cost per person affected falls, adaptation to the new state becomes easier (Frederick and Loewenstein, 1999). Secondly, subjective wellbeing is strongly affected by a person’s treatment relative to the treatment of others (Argyle, 1999, Layard, 2011). Consequently, the negative response to an imposed cost is likely to diminish when it is also borne by a person’s contemporaries. Thirdly, individuals may be prepared to participate in a communitarian activity even if the subjective costs are not diminished so long as others participate. In an analogous decision, individuals may be prepared to contribute a small amount to save the life of a sailor lost at sea even if they are told that the number of people who will make a contribution is so large that, by usual standards, the rescue is not cost effective. Participation in communal activities may be perceived as the role of a citizen rather than something to be assessed by the action of a self interested individual.

Two surveys are reported below which test this hypothesis: that people will allocate resources to low volume, 'cost ineffective' – effective but high cost – services when the costs are shared despite knowing that their decision will reduce total health. The hypothesis implies that the allocation will increase with the number of people sharing the cost. Three subsidiary hypotheses are also investigated. The first – 'normal substitution' – is that as the price of the high cost service rises, the coverage of its costs will fall. The second – 'weak sharing' – is that as the price increases and the opportunity cost to others rises, the proportion of the budget assigned to the high cost patients will diminish, ie the willingness to share is negatively related to the total burden it imposes. The third – 'strong sharing' – is that, over the parameters in the survey, the total share of the budget allocated to the high cost service will increase with the price of the service to offset the effect of the price rise.

In Section 2 below we describe the survey and the tests used to investigate the hypotheses. Results are presented and discussed in the following two sections.

2 Methods

In sum, survey respondents were asked to divide a budget between a small group of patients with a high cost illness (A), and a large number of patients with a low cost illness (B). Treatments were divisible and the quality of life of both groups of patients was directly proportional to the amount of the budget allocated to them. The difference in treatment costs was sufficiently large that expenditures upon the low cost illness, B, were always more cost effective. The symptoms of the two illnesses were identical and were separately evaluated to allow an estimate of the net QALY loss from expenditure upon illness A. The number of patients, B, and the cost of illness A were varied. The study hypotheses were tested by observation of the allocation of resources to illness A.

Survey

Two web-based surveys were administered by a talking avatar to members of the public who were enrolled with a panel company CINT Pty Ltd. The surveys only differed with respect to the order of presentation of the questions and the total budget which was to be allocated. Both surveys commenced with an introduction and overview by the avatar:

> *Hello and welcome, I'll be your guide today… Thanks for participating in this project… your answers will help us understand how the public thinks Medicare should*

allocate its budget between various patients when the cost of treating them varies significantly and it is not enough to give a complete cure to everyone.

Personal details were then obtained and in each survey, respondents assigned to a demographic cohort until the cohort quota was filled. The surveys were then divided into three parts. First, questions were asked to familiarise respondents with relevant health states which were then evaluated on a visual analogue scale (VAS). The second and major part of the survey asked respondents to divide the available budget between the small number of high cost patients (A) and the large number of low cost patients (B) as the relative cost of treatments, the number of patients B, and the total budget varied. In the final part, questions were asked to gain insight into people's reasons for their previous answers but also to determine respondent consistency between the budget allocations and their stated motivations.

Part 1: In the first part of each survey respondents were asked to rank four health states relating to walking and self care. These were taken from the descriptive system of the EQ-5D-5L in which the levels of disability are described as 'slight', 'moderate', 'severe' and 'unable to walk and self care. The ranking exercise was followed by an explanation of a VAS (reproduced in Appendix 1) and respondents were asked to rate the four health state using the VAS. Scores were converted into TTO utilities using a transformation estimated from 3714 paired VAS – TTO observations obtained from interviews for the construction of the AQoL-8D MAU instrument (Richardson et al., 2014). The algorithm is described in Appendix 2 and compared with the algorithms used for the construction of the HUI 2 and HUI 3.

Part 2: The second part of the survey was introduced by the avatar as follows:

Suppose you live in a small town of 1,000 people. Experts advise that next year two illnesses will occur which we will call illness A and illness B. Both illnesses have the same symptoms which are problems with mobility and self-care. Five people will get illness A and 100 people will get illness B. Anyone in the town – men and women of all ages – may get one of the illnesses. You, personally, could be one of these people.

Both illnesses are life threatening and without treatment the patients will die. With some treatment the patients will be saved from death but left with problems with walking and self-care such as dressing, washing and toileting. The more treatment the patient receives the more their health will be improved until the illness is cured. The

> *effect of the treatment, whether it is partial improvement or full cure lasts 10 years after which it will have to be repeated.*
>
> *But here's the catch. Because the causes of the illnesses are very different the cost of treatment for the two illnesses is very different … the government has allocated a fixed budget to treat the two illnesses. But the amount in all the questions that follow is not enough to provide a full cure for everyone. Extra private spending is not possible and no other services will cure the illness. The questions are about how you think the government should distribute its money.*

Respondents were then presented with a series of figures which summarised the parameters of the choices they were to make. One of these is reproduced in Box 1. The descriptions of health states in the figures were aligned with the percentage of cost coverage, the numerical value of which equalled 100 times the utility of the health states. These were obtained from an earlier study which used the same scale and descriptors (Richardson et al., 2015). Therefore, in selecting a level of cost coverage, respondents were selecting the health state utility which would be experienced by patients if they contracted the illness.

The avatar continued:

> *Using the slider you can choose how to divide the money between the two illnesses and therefore how much health to create for each group of patients. Notice that I am talking about the amount of health created which is represented by the size of the blue areas. Moving the handle all the way to the right creates the maximum total amount of health as shown by the large blue area. This is because illness B is cheaper to cure so you can buy the most units of health if you spend all of the funds on illness B. But if you do this the five patients with illness A will get nothing and they all die.*
>
> *Moving the handle all the way to the left creates the minimum total amount of health. You can see this because the total blue area – adding the blue area of A and B together – is much smaller than before. This is because illness A is more expensive to cure so you're buying less units of health when you spend on illness A. When you do this the patients with illness B get less health.*

The budget, the cost of cure and the number of patients B were varied as shown in Table I. The visual representation of the total possible health of patients B expanded and contracted directly with the number of patients. Possible combinations of health were calculated by an algorithm which altered the visual display for both groups as the respondent moved the 'slider'.

The avatar guided respondents through several examples focusing on the trade-off between total health, sharing with A and the sacrifice in total health this would imply. Respondents were then shown the budget, the cost of a full cure for patient A and patient B and asked to allocate the budget. Before the answer was accepted respondents were asked to respond to the following statement:

> *Notice that you are reducing total health so that health can be shared. Please confirm this is what you think should be done. Are you sure you want the XXX patients with illness B to have only XX% of full health so that the 5 patients with illness A can live and have X% of full health?*

The figures XXX, XX and X were inserted by the algorithm.

The two surveys were conducted sequentially with questions in reverse order to mitigate order effects. In survey 1 the initial size of group B was 100. In survey 2 the initial size was 600. The same combinations of costs were used in the two surveys but the budgets differed. In survey 1 the budget was equal to the cost of a full cure for (only) group B (ie less than needed to fully cure both groups). In survey 2 the budget was less than the cost of a full cure for group B except when group B was 100 in which case the questions and budget were identical in the two surveys. The purpose of the replication was to test the importance of order effects.

The level of insurance selected for patients with illness A and the opportunity cost of foregone insurance B and therefore foregone QALYs were calculated for each of the scenarios created by varying the cost of insurance A and the number of patients, B. Regression analyses were used to examine the relationship between the insurance selected and the independent variables including respondent characteristics.

Part 3: Respondents were asked to rate the importance of six possible influences upon their budget allocations. These were (i) the health of group A; (ii) the total health of group A and group B; (iii) the fairness of the distribution between the two groups; (iv) B's loss of health; (v) preserving hope for group A; (vi) avoiding terrible health states. The importance questions were followed by four statements to be rated on a scale from 'strongly agree' to 'strongly disagree'. These probed respondent's attitudes towards the provision of high cost services.

Two edit criteria were used to remove results which indicated a lack of understanding of questions or serious inconsistency. The first was that health states were ranked in the correct order as dictated by the construction of the EQ-5D-5L. (The interchange of ‘unable to walk’ and ‘severe problems’ was accepted). The second criterion was that the numerical values assigned to these health states were also in the correct order. Results below were re-estimated including the edited cases and reported in an appendix.

The survey was approved by the Monash University Human Research Ethics Committee Approval ID: CF15/411 – 2015000201.

3 Results

A total of 702 respondents completed the questionnaire: 353 in survey 1 and 349 in survey 2. Of these 38 percent failed one of the two edit criteria leaving the sample of 432 which was made up of 221 in survey 1 and 211 in survey 2. Appendix 4 reports the composition of deleted cases and re-estimates results with their inclusions. Demographic characteristics of the main sample are reported in Table II. Despite the substantial editing the final sample closely resembles the demographic characteristics of the Australian public which is shown in the final line of the table. The sample was skewed towards those with higher education.

VAS scores for the two sets of health states and the estimated TTO utilities are reported in Table III. For the top two states – slight and moderate problems – the average VAS scores were 4 and 3 percentage points lower than those obtained in the earlier survey which were incorporated in the figures. The lower health state utilities were identical to the earlier estimates.

The percentages of the full cost of care allocated to patients A and B in the two surveys are given in Table IV. Results indicate that the order of presentation of questions had a small but significant effect. When there were 100 patients, identical questions were asked but in reverse order. When P_A=\$2,000, survey 1 respondents assigned 78.3 percent insurance to patients A – 4.3 percent less than respondents in the second survey. The difference increased to 8.9 percent when P_A=\$20,000. Differences are significant at the 1 percent level. The result suggests that presenting the most dire scenario for patients A first – P_A=\$20,000 – parenthesised the need to share and that this influenced subsequent responses. Subsequent differences between survey results are attributable to this order effect but also to the lower budget in survey 2.

Notwithstanding differences between the two surveys the results are consistent with respect to the main study hypothesis. In all scenarios there was a significant allocation of resources to services for illness A despite these services being cost ineffective as discussed below. Increasing the number of patients B, who shared the opportunity cost of services for A, increased the coverage of illness A in all but two cases. The exceptions occur in survey 2 when there are 300 patients B and the cost of A is $2,000 and $5,000. The result may be explained by the reduction in the budget in survey 2 in these cases. However, an order effect may have contributed to the result. In survey 2 the 'start point' was the most dire scenario for patients A; the budget was 17 percent lower relative to full cost and the price 10 times higher than the start point for survey 1. This resulted in a significantly lower allocation to patients B than occurred in the corresponding scenario in survey 1 (74.8 versus 89.4 percent of the full cost of B). The difference may have created greater willingness to impose costs upon patients B, an embedding effect which may explain the lower share allocated to B when budgets were equal (n=100).

Consistent with the first subsidiary hypothesis – 'normal substitution' – the ten fold increase in the price of A led to a reduced cover of between 66 percent (survey 1, 100 patients B) and 36 percent (survey 2, 600 patients B). Figure 1a and 1b plot the average percentage coverage of A against its price. To highlight the order effect, results from survey 2 for 100 patients B are shown as a dotted line beside the results from the same question in Figure 1.

Figure 2a and 2b plot the percentage of the total budget allocated to patients A as the price rises. The corresponding data are reported in Table V along with the opportunity cost which is imposed upon patients B, measured as the reduction in utility per patient. The results are inconsistent with the second, but consistent with the third subsidiary hypothesis. Increasing price is associated with 'strong sharing' – an increasing, not decreasing, percentage of the budget allocated to A. The results also highlight the significant effect of the ordering of questions. The percent of the budget allocated to patients A and the corresponding cost to B, is 8.9 percentage points higher in survey 2 when the most budget constrained scenario was presented first (P_A=$20,000; B=100).

Each of the scenarios implies an allocation which does not maximise QALYs. The smallest budget allocation to patients A was 1.5 percent (Table V, Survey 1, Price=2,000; n=600). From Table IV this corresponds with a utility of 0.89 for patients A. Consequently, each year the five patients would obtain a total of 5 x 0.89 = 4.45 QALYs. From Table IV this required a reduced coverage of illness B of 0.015 for the 600 patients which implies a total loss of 9 QALYs or twice the gain by patients A. Table VI reports the QALY loss from each scenario. The reduced budget in survey 2 (B=300, 600) is associated with a significant increase in the net loss reflecting the greater opportunity cost imposed upon B to maintain sharing in these cases. The same table reports the QALY loss as a percentage of the potential QALYs: the QALYs which would be obtained by following the QALY maximising strategy of allocating all resources to illness B. As above, this indicates that as the cost of A rises, QALYs sacrificed to maintain sharing increases.

Regression Results: Table VII reports results of regression analyses in which the percentage of insurance, A, is the dependent variable. Except where shown, results are significant at the 0.1 percent level. The price of A and the number of patients, B, have the predicted negative and positive signs respectively in all regressions. In regression (2) the inclusion of the opportunity cost of A, (OC), defined as the reduced percentage coverage of B increases the magnitude of both coefficients. The positive coefficient indicates that, contrary to initial expectation, a higher OC is associated with greater, rather than lesser, cost coverage of A. OC is not highly correlated with other variables and the coefficient does not appear to be artefactual. Rather it suggests that causation is not from high OC to low cost cover of A (implying a negative coefficient) but from the selected cost cover of A to the consequential opportunity cost for B. Non linearity of this effect was tested in regression 3 by the inclusion of a quadratic term. The small coefficient of -0.003 is significant at the 5 percent level implying a decreasing marginal relationship with the dependant variable.

Regression 3 also included a variable, TC(B) which measured the total cost to all of the patients B (ie per patient cost times the number of patients. The coefficient of -0.03 is significant at the 5 percent level and supports respondents response to subsequent questions that total cost was an important consideration when allocating the budget (Table VIIII below). A dummy variable ‘Survey 2’ was included in each of the regressions to identify unaccounted differences between the surveys. Only one of these, regression 4, is reported. The magnitude of the coefficient (-21.5) is implausibly large and attributable to multi-collinearity. A similar coefficient was found in other regression equations which included this term.

Rating Questions: Tables VIII and VIIII report results from respondents’ ratings of the importance of different arguments when they made decisions (Table VIII) and their rating of general statements concerning the allocation of resources (Table VIII). With two exceptions, results from the two surveys did not differ at the 10 percent level and only pooled results are reported. From Table VIII, three considerations were nominated as most important, namely, the avoidance of terrible health states, fairness in the distribution of health and the amount of total health. The two statements focusing upon a single group – the health of group A and the loss of health to group B – were less important, as was the preservation of hope. From Table VIII there was majority agreement with the proposition that a small decrement in the coverage of care for the majority was acceptable as a means of covering the cost of rare but expensive illnesses and that priority should be based primarily upon the severity of illness rather than cost. A near majority of respondents rejected 'basic only' treatment for expensive illnesses and agreed that the Medicare levy should be increased to cover rare but costly illnesses.

Appendix 4 re-estimates the main results in Table IV using both the unedited database and those cases which were deleted. The overall pattern of results is unaltered in the full database. Deleted cases displayed less variation with price suggesting lesser discrimination with lesser attention to the questions.

4 Discussion

Therapies for rare and ultra rare disorders (URD's) – 'orphan disorders' – are commonly too expensive to satisfy cost effectiveness criteria despite their – by definition – low prevalence. Nevertheless regulations have been designed to encourage the development of these treatments (Meekings et al., 2012, Melnikova, 2012, Woodock, 2012) and there is currently a mismatch between R & D policies and cost effectiveness based policies for the funding of health services (Schlander et al., 2014). In principle these policies may be reconciled as economic theory has always recognised the possibility of a trade-off between equity and efficiency. However, to date there has been limited evidence that the public would consider 'equity' to extend to the funding of URDs with at least one study of Norwegian doctors finding that rarity per se was not considered grounds for special treatment (Desser, 2013). An exception was an earlier study by the same author (Desser et al., 2010) which found that 80 percent of 1479 Norwegians endorsed the statement that 'all should have equal access to health regardless of costs'. However the study authors were 'reluctant' to accept this evidence as support of rarity as the basis of equity.

Equity-efficiency trade-offs have usually been justified by factors related to benefits: QALY weights to account for age, severity or proportional shortfall. However trade-offs have been accepted in the absence of these factors. For example, in Australia, the Pharmaceutical

Benefits Advisory Committee (PBAC) which is responsible for the assessment of drugs, accepts that ‘cost ineffective’ drugs may be funded because of the Rule of Rescue (Littlejohns and Rawlins, 2009 p. 118, McKie and Richardson, 2003). In their guidelines this is described by three characteristics, viz, that no alternative treatment exists; that the relevant condition is severe, progressive and expected to lead to premature death; and that the condition applies to a very small number of patients (Cookson et al., 2007). These criteria define circumstances which are similar to those considered in the present paper which suggest that the findings of the present survey are consistent with the intuition of at least some decision makers.

In contrast with the Norwegian study cited above, the present surveys did not focus upon rarity per se but upon the consequences of rarity for the costs borne by others, when costs are shared. Results support the hypothesis that people are willing to share the high cost of low volume services. An important conclusion is that they are willing to do so with full knowledge of the consequences of their decisions for total health. Respondents were asked to confirm their understanding of this after each question. Results in Table VIIII indicate that respondents took account of the opportunity cost of their choices but were concerned by ‘terrible health states’ and the fairness of the distribution of health. From Table VIII respondents explicitly agreed that it is ‘OK to reduce services to the majority by a little to cover the cost of very expensive services needed by the few people with rare illnesses’ and that ‘if services for severe illnesses are very costly, the cost should be shared across the whole community’. These, and other stated opinions are consistent with the values postulated in the sharing hypothesis.

The effects found in the survey are not marginal. The price of A was raised to 20 times the price of B but this increased sharing to the point where a maximum of 37 percent of possible QALYs were foregone to maintain services to the high cost patients. Increased cost was nevertheless associated with a reduced coverage of the high cost service but the reduction depended upon the relative number of patients sharing the cost. From Table IV the ten fold increase in cost in survey 1 reduced coverage of A by 52 percentage points (from 78 to 26 percent) when the number of patients B was 20 times greater than A but by only 25 percentage points (from 89 to 64 percent) when the number of B was 120 times A and the average opportunity cost per patient B was therefore lower.

Results from a web-based survey are subject to a number of methodological caveats. There is no quality control at the point of delivery and a significant proportion of respondents are known to provide careless or frivolous answers. For this reason the survey was subject to the severe editing described earlier and the removal of 38 percent of cases. The re-estimation of results including the edited cases did not, however alter the main conclusions (Appendix 4). Nevertheless questions were cognitively demanding and responses were unavoidably subject to potential bias from the framing of the questions. As discussed, the order of presentation of questions affected responses but the effect was small relative to the main experimental effects. An additional bias was introduced by calibrating the health states using utility data which, in the event, differed from respondent's own assessment by 3-4 percent for the best health states. This reduced the visual representation of the total loss of utility for B and, potentially, inflated the willingness to share with A. Offsetting this effect, the estimated net QALY loss retained the initial utility scores which reduced the QALY loss to B. However these effects are small in relation to the overall results and the study was specifically designed with a very broad range of parameters to reduce the sensitivity of the main conclusions to unavoidable survey error.

Simple generalisation of the survey creates an apparent anomaly. As the number sharing the cost of a single rare disease rises from 600 to over 1 million the opportunity cost to patients B would approach zero and disease cost would cease to affect allocation decisions. In part the anomaly is a reflection of the study design which retained the same small number of patients A to simplify scenarios and to emphasise that the budget allocation to illness A benefited a very small number of patients. The actual number of patients who could benefit from high cost treatment is, of course, significantly greater and variable with the inclusion criteria. To adjust health policy to take account of sharing would require a reinterpretation of the survey results so that A represented a broad category of high cost low volume illnesses and B represented illnesses where coverage could be reduced. As found in the survey, as the average cost to patients in the second category, B, increased the preferred coverage of patients in the first category, A, would fall. Consequently, the addition of new high cost services to category A would be limited by the opportunity cost to B.

The challenge, in practice would be to identify patients in the two groups, A and B and to determine an algorithm which incorporates the socially preferred level of cost sharing. In principle, this task – triaging services into those to be included or excluded from insurance cover – is the same as the task presently carried out in CEA which is summarised in equation 1.

$$\text{Cost/QALY} \leq T \qquad \ldots \text{ equation 1}$$

Cost per QALY must be less than a threshold, T, which may be selected to achieve a target budget. The algorithm may be adjusted to equation 2 to take account of sharing and severity.

$$\text{Cost/QALY} \leq wT \qquad \ldots \text{ equation 2}$$
$$w = f(Sev, N)$$

where w is a weight which is a function of the disease severity, Sev, and the number of patients N who have the diseases whose costs will be shared. As severity increases, w will decline; as N increases and the opportunity cost of sharing increases, w will rise. An algorithm satisfying these conditions is illustrated in Appendix 3. With maximum severity and an illness affecting 0.001 percent of the population the algorithm produces a weight of 20. The 'opportunity cost' is that for illnesses affecting 1 percent of the population which have low severity – a pre-treatment utility of 0.8 – the weight is 0.4, implying the transfer of resources from this group to high cost illnesses with small patient numbers.

A potential problem with the application of these results is that they assume the possibility of partial, not complete provision of the high cost services. When therapies are indivisible the average cost may exceed a revised threshold based upon severity and discounted cost. There is an analogous problem with current theory if a cost effective therapy is applicable to so many patients that the budget is exceeded. However, the practical problem is a separate question from how an informed public would like rare but expensive services to be evaluated when this was feasible and the evaluation framework which could incorporate these preferences.

Empirical results are implausible unless there is a plausible explanation for them and particularly when the results are inconsistent with established theory. The willingness to share implies a deviation from the utilitarian values which are the basis of welfare theory. In this, the maximisation of utility is a consequence of utility maximisation by each individual. In the simple market envisaged by welfare theory the individual receives both costs and benefits from a service. Ensuring that the utility from the latter is greater than or equal to the utility from the former is therefore a compelling and rational evaluation criterion. However the group of beneficiaries of a health service is distinct from the group sharing the costs. There is not a compelling reason to directly compare costs and benefits as distributive considerations become important. When sharing results in a low cost to any one individual and participation in a communal activity is seen as a duty or as the normal consequence of membership of the community, utilitarianism may cease to be the preferred or only basis for allocating resources. . Evidence from the present survey supports this conclusion.

Social preferences may be ignored if they imply ethically unacceptable consequences. The survey results may be seen to imply horizontal inequity between patients who would receive treatment if their illness was rare and costly but not if the illness was common and cheap. However the survey results do not imply this conclusion. Costs were the result of incremental reduction from full health, for the larger group, B. The benefit A was an improvement from death or very poor health. Nevertheless it may appear ethically unacceptable to allocate dollars to a patient when the additional utility from treatment is less than the utility which might be obtained by allocating it to another patient. However the fact that population preferences do not conform to a particular ethical system – consequentialist utilitarianism – does not represent a strong argument for dismissing them. Preferences may be 'laundered' but the case for this is strongest when preferences are abhorrent or ill informed. The present results are in neither of these categories. Respondents were asked to confirm their answers. To the extent that the values revealed by these answers reflect a concern for others they are consistent with defensible 'communitarian' values and with the significant ethical literature which argues for prioritising the worst off members of society (Alkire and Santos, 2013, Clark and Qizilbash, 2008, Mitchell et al., 2015, Rawls, 1971).

5 Conclusions

A willingness to share is a fundamental characteristic of a communal enterprise. However, at present sharing per se has no part in either the theory or practice of economic evaluation. Evidence from the present survey suggests that when patient numbers are small and the average cost to those who share the cost is small, a well informed public is likely to support the funding or part funding of some services which are presently considered 'cost ineffective' because of their high cost per patient. Incorporating these preferences in a workable algorithm for the allocation of the health budget is problematical and the present results do not resolve the problem. However the fact that the structure of public preferences creates a problem for the funding formula cannot be taken as evidence that preferences do not have this structure. In general terms a public preference for sharing small volume high cost services increases the importance of the service efficacy and of the severity of a disease in the algorithm but does not eliminate cost, as the average impact upon those bearing the cost varies with the total cost to the budget of the services.

Table I Survey parameters

Both surveys				Survey 1		Survey 2	
Number of patients		Cost of cure					
Group A	Group B	A $000	B $000	Order delivered	Budget ($000)	Order	Budget
5	100	20, 15, 10, 5, 2	1.00	1	100	3	80
5	300	20, 15, 10, 5, 2	1.00	2	300	2	250
5	600	20, 15, 10, 5, 2	1.00	3	600	1	500

Table II Demographics – percentages

	Age groups (percent)								Educational level			Totals
		18-24	**25-34**	**35-44**	**45-54**	**55-64**	**65+**	**Total**	**High school**	**Dip/Trade/TAFE**	**Uni**	**n**
Survey 1	Male	9.4	14.0	21.5	21.5	15.9	17.7	100	27.1	29.9	43.0	107
	Female	15.8	13.1	15.8	21.1	18.4	15.8	100	19.3	43.0	37.7	114
	Total	12.7	13.6	18.5	21.3	17.2	16.7	100	23.1	36.7	40.2	221
Survey 2	Male	14.4	17.1	21.6	16.2	12.7	18.0	100	23.4	19.8	56.8	111
	Female	20.0	12.0	17.0	22.0	11.0	18.0	100	22.0	29.0	49.0	100
	Total	17.1	14.7	19.4	19.0	11.9	18.0	100	22.7	24.2	53.1	211
Total	Male	11.9	15.6	21.6	18.8	14.2	17.9	100	25.2	24.8	50.0	218
	Female	17.8	12.6	16.4	21.5	14.9	16.8	100	20.5	36.5	43.0	214
	Total	14.8	14.1	19.0	20.1	14.6	17.4	100	22.9	30.6	46.5	432
	Total	12.4	19.0	17.6	17.6	14.8	19.2	100				
Australia	Total	11.0	19.3	18.2	17.5	15.0	19.0	100				

Source: ABS (2015) http://stat.abs.gov.au/

Table III VAS and Estimated TTO utilities

Health state	TTO mean(1)	VAS mean	SD	Max	Min
Slight problems with walking and self-care	0.90	76	12	100	30
Moderate problems with walking and self-care	0.77	59	12	95	20
Severe problems with walking and self-care	0.53	44	12	96	10
Unable to walk and self-care	0.29	19	12	85	0

(1) $(1\text{-TTO}) = (1\text{-VAS})^{1.62}$

Source: Appendix 2

Table IV Percent of full cost allocated to Patients A, Patients B

Survey	Number of patients B	Budget	% of full cost given to A					Max - Min	% of full cost given to B					Max - Min
			Price A						Price A					
			2000	5000	10000	15000	20000		2000	5000	10000	15000	20000	
1	100 (1)	100	78.3	68	43.7	34.2	25.6	52.7	92.2	86.4	78.1	74.4	74.4	17.8
2	100 (1)	100	82.6	68.2	51	41.1	34.5	48.1	91.7	82.9	74.5	69.2	65.5	26.2
1	300	300	87.6	78.6	67.1	59.4	52.3	35.3	97.1	93.5	88.8	85.1	82.6	14.5
2	300	250	74.5	63.9	56.6	49.3	45.2	29.3	80.8	78	73.9	71	68.2	12.6
1	600	600	89	83.9	75.6	69.6	63.5	25.5	98.5	96.5	93.7	91.3	89.4	9.1
2	600	500	79.1	71	63.8	59.1	50.7	28.4	82.2	80.3	78.2	75.9	74.8	7.4
1	n(600)/n(100)		1.14	1.23	1.76	2.04	2.28		1.07	1.12	1.2	1.23	1.2	

Table V Distribution of the budget and the excess burden for A (1)

Survey	Number of patients B	Budget	% Budget allocated to patients A					Max/ Min	Opportunity Cost per patient B (100-%B)					Max/ Min
			Price A						Price A					
			2000	5000	10000	15000	20000		2000	5000	10000	15000	20000	
1	100	100	7.8	13.6	21.9	25.6	25.6	17.8	7.8	13.6	21.9	25.6	25.6	3.28
2	100	100	8.3	17.1	25.5	30.8	34.5	26.2	8.3	17.1	25.5	30.8	34.5	4.15
1	300	300	2.9	6.5	11.2	14.9	17.4	14.5	2.9	6.5	11.2	14.9	17.4	2.00
2	300	250	3.0	6.4	11.3	14.8	18.1	15.1	19.2	22.0	26.1	29.0	31.8	1.66
1	600	600	1.5	3.5	6.3	8.7	10.6	9.1	1.5	3.5	6.3	8.7	10.6	7.1
2	600	500	1.6	3.6	6.4	8.9	10.1	8.6	17.8	19.7	21.8	24.1	25.2	1.42

Notes

(1) In all cases the full price of B was $1,000. In 4 of the 6 cases the budget is 1,000 times the number of patients B, n(B). (Survey 1, n=100, 300, 600; Survey 2 n=100). In these cases the opportunity cost per patient B, measured as a percentage reduction in utility, is numerically equal to the percent of the budget allocated to A.

Table VI Net QALY loss per annum

		QALY Loss					Percent possible QALYs lost					
		Price A					Price A					
Survey	**Number of Patients B**	**2000**	**5000**	**10000**	**15000**	**20000**		**2**	**5**	**10**	**15**	**20**
1	100	-4.1	-10.2	-19.8	-19.9	-24.3	100	4.1	10.2	19.8	19.9	24.3
2		-4.2	-13.7	-23.0	-28.7	-32.7		4.2	13.7	23.0	28.7	32.7
1	300	-4.3	-15.6	-30.2	-41.7	-49.6	300	1.4	5.2	10.1	13.9	10.9
2		-53.6	-62.8	-75.5	-84.5	-92.8		21.4	25.1	30.2	33.8	37.2
1	600	-4.6	-16.8	-34.0	-48.7	-60.4	600	0.8	2.8	5.6	8.1	15.5
2		-104.0	-114.1	-128.8	-141.6	-148.1		20.8	22.8	25.8	28.3	29.6

Table VII Regression results: Percent cover of cost of illness A as dependent variable

	Regression number			
Independent variables	**1**	**2**	**3**	**4**
Price A	-2.0	-2.7	-2.09	-3.09
n(B)	0.04	0.06	0.06	0.05
OC		0.91	1.09	1.34
OC^2			-0.003	
TC(B)			-0.03**	
Dum 18-24[(2)]	4.3	2.9*	2.8*	4.66
Dum 25-34	ns	ns	ns	1.80*
Dum 35-44	ns	ns	ns	2.35
Dum 55-64	ns	2.2*	2.1**	2.02**
Dum 65+	ns	ns	ns	ns
Male	-3.2	-2.7	-2.6	-1.69
Survey 2				-21.5
Constant	70.7	56.4	54.7	62.1
R^2	0.31	0.42	0.42	0.49
n	6479	6479	6479	6479

Notes
(1) Unless indicated, results are significant at 0.1 percent (0.000) level

* Significant at 1 percent; ** Significant at 5 percent; n(B): number of patients B

OC: Opportunity cost of funding A: the percentage reduction in the funding of B.

$(OC)^2$: OC*OC

Dum X: Dummy variable = 1 in age range x; otherwise zero

Male: Dummy vary = 1 for males; 0.0 for females

Survey 2: Dummy variable =1 for survey 2; 0.0 for survey 1.

Table VIII Agreement with Statements[1]

	Mean	se	Strongly agree	Strongly disagree	Z score
It is OK to reduce services to the majority by a little to cover the cost of very expensive services needed by the few people with rare illnesses	3.54	0.05	62.5	19.0	13.02
It is not OK to provide the few patients requiring very expensive services with only basic low cost care even if they are left in poor health because Medicare has a limited budget and can't pay for everything	3.40	0.05	48.6	23.6	7.65
The Medicare levy should be increased to cover very high cost care needed by a small number of patients	3.25	0.05	46.5	25.4	6.46
The severity of illness, rather than the cost of treatment, should determine priority. If services for severe illnesses are very costly the cost should be shared across the whole community	3.72	0.05	64.4	13.2	15.4

Notes

(1) The full distribution of responses is given in Appendix 5

(2) Category (1) and (2) on a 5 category response scale

(3) Category (4) and (5) on a 5 category response scale

(4) 'Not' inserted to align response types in a single column

Table VIIII Importance while answering questions (1)

1 = not important; 5 = very important (n=432)

	Mean	**se**	**Very important/ important(2)**	**Not at all important/ unimportant(3)**	**2 score**
The health of patients in Group A	3.65	0.049	53.2	10.4	13.5
The total amount of health (the area shaded blue)	4.17	0.043	78.5	3.9	22.3
Fairness in the distribution of health	4.19	0.044	77.4	5.1	21.6
The loss of health in Group B by giving money to Group A	3.80	0.046	63.4	7.4	17.2
Preserving hope for Group A	3.70	0.053	59.9	13.4	14.2
Avoiding terrible health states	4.19	0.047	76.4	5.5	21.2

Notes
(1) The full distribution of responses is given in Appendix 4

(2) Category (1) and (2) on 5 percent scale

(3) Category (4) and (5) on 5 percent scale

(4) ‘Not’ inserted to align ‘agreement’ in a single column

Box 1 Visual aid for budget allocation

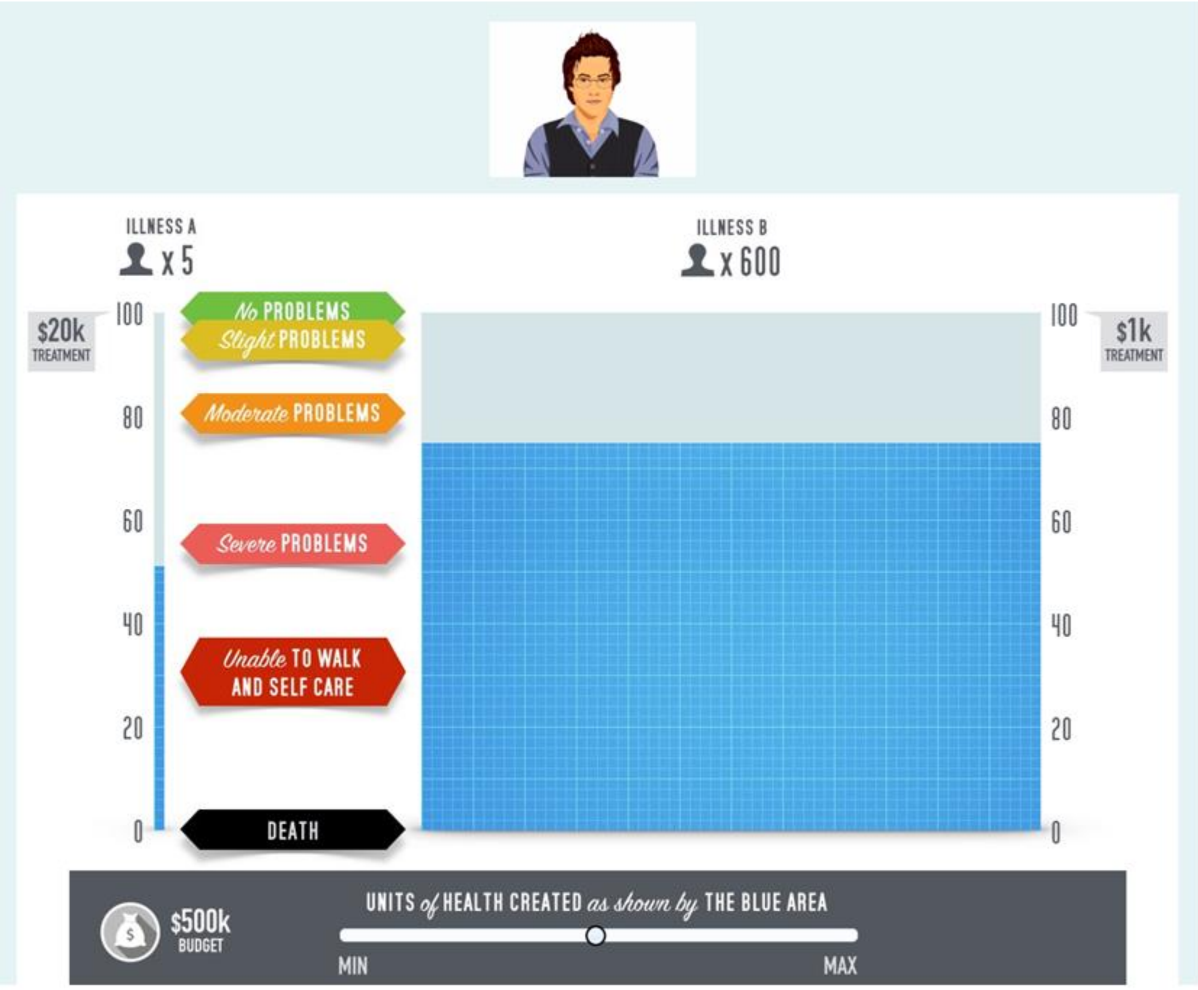


Figure 1 Percent coverage of total cost of illness A by Price A and size of Group B

Fig 1a Survey 1, n=100, 300, 600; survey 2 n=100

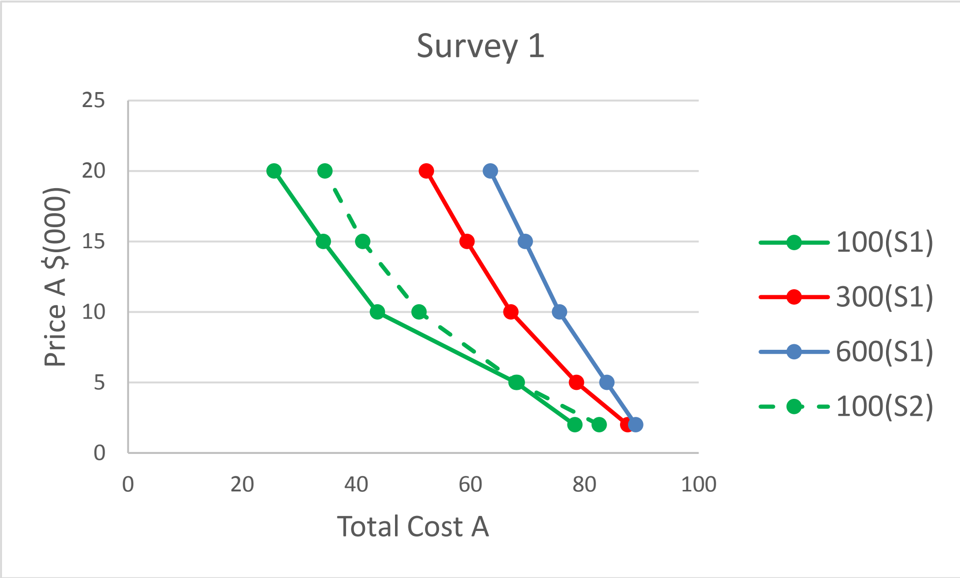


Fig1b Survey 2, n =300, 600

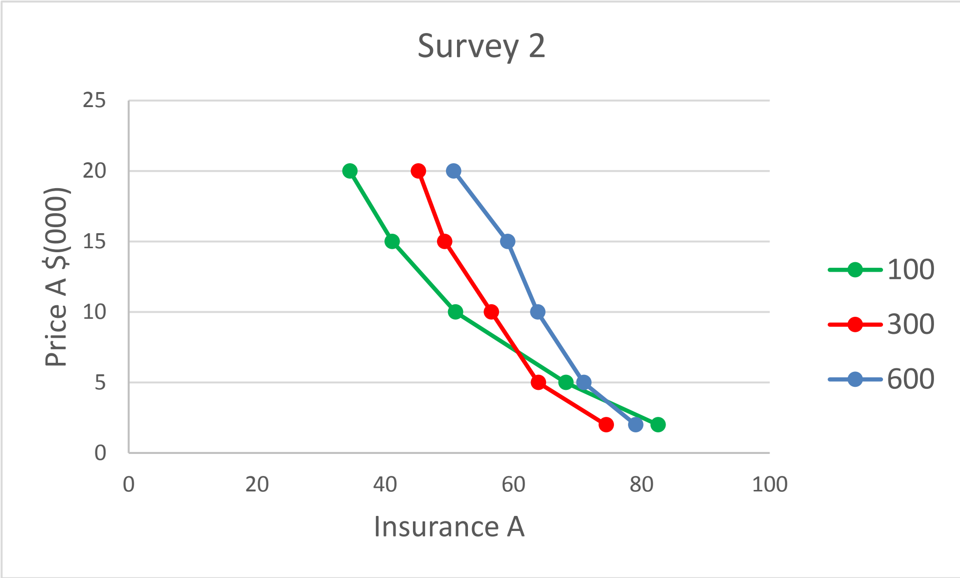


Figure 2 Percent of budget allocated to patients A as price varies

Fig 2a Survey 1

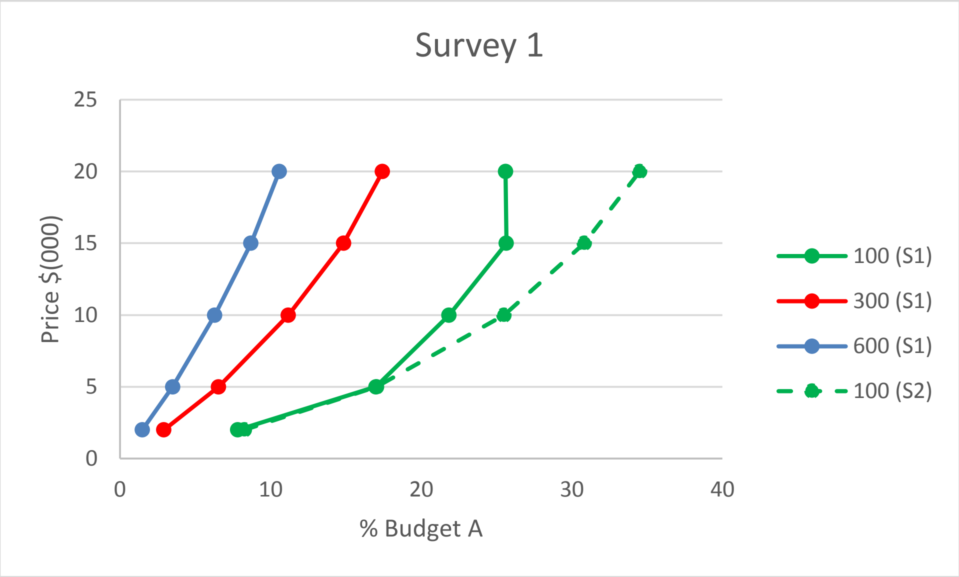
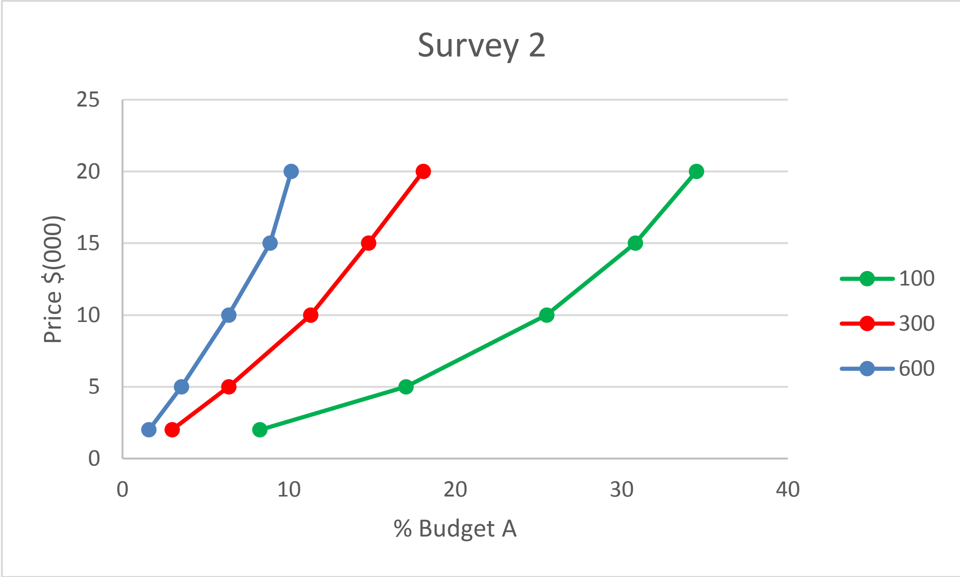


Fig 2b Survey 2



Appendix 1 Description of the VAS (rating scale)

The text and figures below are an edited extract from the online questionnaire. The visual aid and question for only one of the two illnesses is reproduced. The second visual aid and question differed only with respect to the health state descriptors.

Introduction to the Rating Scale:

Now we would like you to evaluate the health states that have been used (in the survey) on a rating scale such as the one shown in Box A.1. This is a way of measuring how strongly people feel about different things.

On this scale, 0 represents Death and 100 represents Full Health.

There are no right or wrong numbers for a health state. Rather the distance between points on the scale shows how strongly you would feel about these health states.

For example if you gave three health states, A, B and C a score of 50, 60 and 70 it would mean that you felt the improvement from A to B was about as much – or as important for you – as a move from B to C.

Similarly a move from death to A (score 50) would be about as important for you as a move from A to full health.

Box A.1 Evaluating Mobility and Self Care

Question:

On our scale 'Full Health' does not mean perfect health but 'no problems with walking, self care or usual activities: it is good, not perfect health'.

Please indicate using the scale how good or bad these health states are, in your opinion. On this scale 0 represents death and 100 represents full health.

Write your answers in the highlighted boxes beside each health state.

Health state 1

I have *slight* problems with
- Walking and self-care

Health state 2

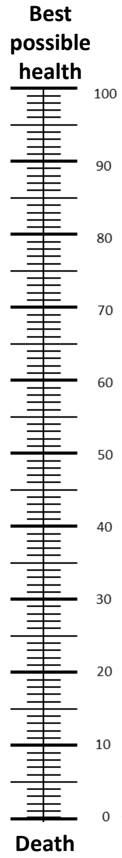
I have *moderate* problems with
- Walking and self-care

Health state 3

I have *severe* problems with
- Walking and self-care

Health state 4

I am unable to
- Walk about or self-care



Appendix 2 Transforming VAS values to TTO utilities

The transformation from VAS values to TTO utility used a relationship derived during the construction of the AQoL-8D utility algorithm (Richardson et al., 2014). A total of 162 health states describing the 8 dimensions of the AQoL-8D were evaluated during an interview using both a VAS and a TTO. The sample of 670 individuals interviewed included 323 patients undergoing treatment and 347 demographically representative members of the Australian public. On average each was asked to rate 5.5 health states giving a total 3,714 observations or an average of 23 observations per health state. The 162 average results were used to estimate a number of regression models. The most successful of these was equation A 2.1 below.

$U = 1-(1-V)1.62$ …equation A 2.1

By comparison, in the construction of the HUI 2 Torrance et al. (1996) used 4 points derived from average data to fit the transformation function A 2.2.

$U = 1-(1-V)2.29$ …equation A 2.2

where U was estimated using a standard gamble and V employed a VAS.

For the construction of HUI 3 Feeny et al. (2002) employed three marker states between full health and death. Two functions were estimated; one for those where the worst health state was worse than death and one for where it was better than death. His selected transformation for the larger, former group is given by A 2.3.

$U = VAS0.559$ …equation A 2.3

where U was measured as a standard gamble and VAS on a ‘feeling thermometer’.

The difference in predicted utilities from these functions is illustrated below. They imply similar results but with a greater concentration of utilities using the Torrance formula at the top of the scale and a greater inflation of utilities using the Feeny formula at the bottom of the scale. Differences may be explained, to an unknown extent, by the different survey methodologies employed and by the use of a standard gamble rather than the time trade-off as in the estimation of AQoL-8D utilities.

Table A.2.1 Comparisons of 3 VAS-Utility transformations

VAS	0.0	0.1	0.2	0.4	0.6	0.8	0.9	1.0
Utility								
Torrance[(1)]	0.0	0.21	0.40	0.69	0.88	0.97	0.99	1.0
Feeny[(2)]	0.0	0.28	0.41	0.60	0.75	0.88	0.94	1.0
Richardson[(3)]	0.0	0.16	0.30	0.56	0.77	0.93	0.98	1.0

Notes

(1) Torrance (1996); (2) Feeny (2002); (3) Richardson (2014)

Appendix 3 An illustrative decision algorithm

Equation A3.1 is an alternative formulation of equation 2 in which the threshold, T, remains unchanged but severity and the patient numbers create a variable QALY weight, w*=w-1.

$$\text{Cost/w*QALY} \leq T \qquad \text{… equation A3.1}$$

Table A3.1 illustrates a hypothetical set of such weights which would result from the weight function w*=(Sevα) (N-β) and the illustrative parameters are α=0.5, β=0.434. The illustrative weights are relative to the importance attributed to a life saving service (Sev=1) which affects 1 percent of patients (N=1). As the severity of the initial health state, Sev, decreases from 1.0 (imminent death) to 0.2 (an initial health state utility of 0.8), the severity weight for services affecting 1 percent of the population decreases from 1.0 to 0.45. As the number of patients decreases from 10 to 0.001 percent of the patient population, the sharing weight increases by a factor of 20/0.37=54. The multiplicative algorithm therefore implies a weighting which is 56/0.45=318 times greater for services which affect only 1 in 100,000 patients who are facing imminent death than for services which affect 10 percent of patients who are in relatively good health.

Table A.3.1 Hypothetical weights incorporating severity and sharing

Percent of population		**Initial HS: Sev 1.0**	**0.8**	**0.6**	**0.4**	**0.2**
		Sev: .5 1.0	**0.89**	**0.72**	**0.63**	**0.45**
N	$N^{-.434}$	$(Sev^{.5})(N^{-.434})$				
0.001	20	20	17.8	15.4	12.6	8
0.01	7.3	7.3	6.5	5.6	4.6	2.9
1.0	1	1	0.89	0.72	0.63	0.45
10	0.37	0.37	0.33	0.27	0.23	0.17

Appendix 4 The effect of editing

Table A.4.1 reports the demographic characteristics of those who failed one of the edit criteria. The failure rate is greatest at the age group 25-34 but this did not result in significant under-representation of this cohort.

From Table A.4.2 and the corresponding Figure A.4.1 the responses of those deleted followed the same pattern as the edited responses. Patients A were always allocated resources and the share declined with the price of A. However the decrement as price rose which is consistent with less discrimination by respondents and less well considered answers.

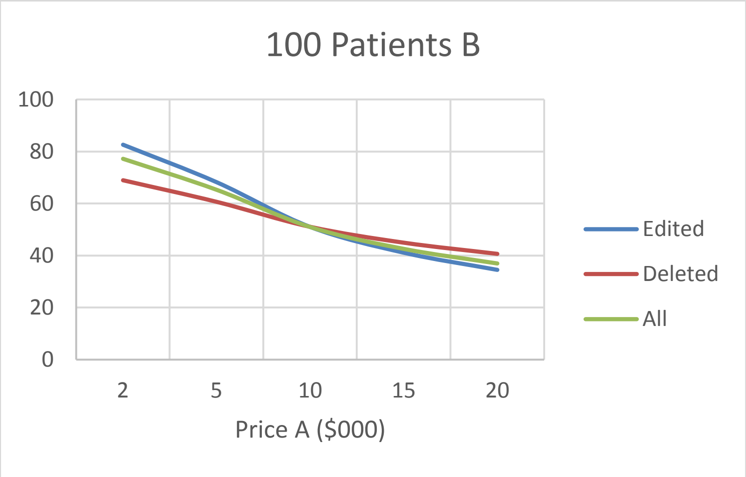
Table A.4.1 Participants deleted from the final analysis n=138

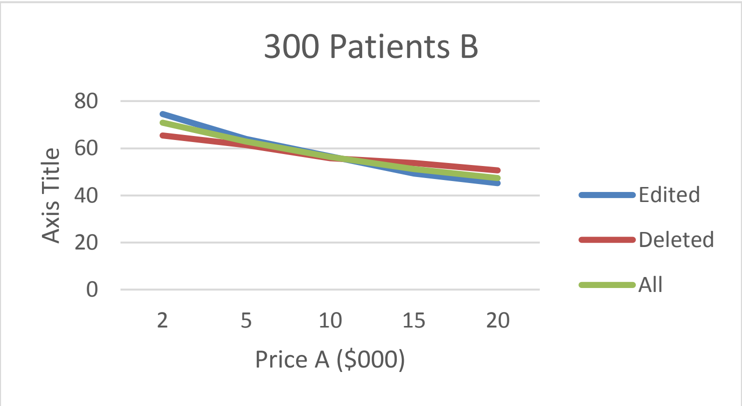
	18-24	25-34	35-44	45-54	55-64	65+
Male	4.3%	10.1%	8.0%	10.1%	9.4%	9.4%
Female	2.2%	13.8%	8.7%	7.2%	9.4%	7.2%

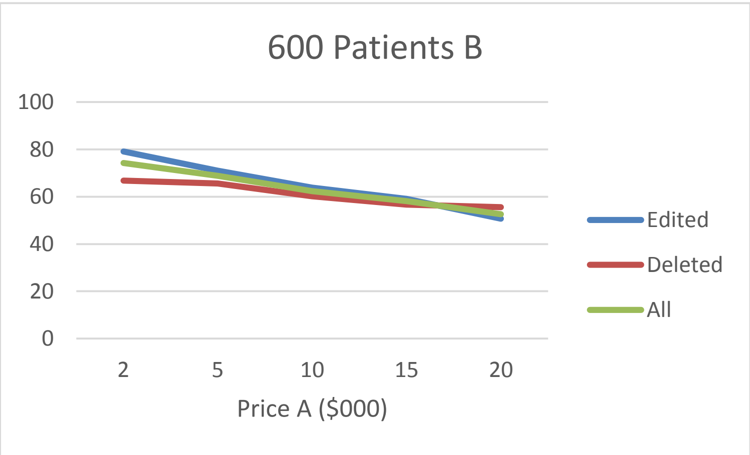
Table A.4.2 Comparison of edited, deleted and all data. Percent of full cost allocated to patient A

Number of patients B	Budget	Sort 2 survey	n	% of full cost given to A					Max - Min
				Price A					
				2000	5000	10000	15000	20000	
100	100	Edited	211	82.6	68.2	51	41.1	34.5	48.1
		Deleted	138	69	60.7	51.1	45	40.7	28.3
		All	349	77.2	65.3	51	42.6	37	40.2
300	250	Edited	211	74.5	63.9	56.6	49.3	45.2	29.3
		Deleted	138	65.4	61.3	55.8	53.8	50.6	14.8
		All	349	70.9	62.9	56.3	51.1	47.3	23.6
600	500	Edited	211	79.1	71	63.8	59.1	50.7	28.4
		Deleted	138	66.8	65.5	60.1	56.7	55.5	11.3
		All	349	74.2	68.8	62.3	58.1	52.6	21.6

Figure A.4.1 Percent coverage of cost of A







Appendix 5

Table A4.1 Agreement: Answer to the question 'Please indicate on the scale how you feel about each of the following statements' (n=432)

	Mean	Std deviation	Std error of mean	% answer 1 (not at all important)	% answer 2	% answer 3	% answer 4	% answer 5
It is OK to reduce services to the majority by a little to cover the cost of very expensive services needed by the few people with rare illnesses	2.44	1.04	0.05	16.0	46.5	18.5	15.5	3.5
It is OK to provide the few patients requiring very expensive services with only basic low cost care even if they are left in poor health because Medicare has a limited budget and can't pay for everything	3.40	1.07	0.05	2.1	21.5	27.8	31.7	16.9
The Medicare levy should be increased to cover very high cost care needed by a small number of patients	2.75	1.10	0.05	11.3	35.2	28.0	18.5	6.9
The severity of illness, rather than the cost of treatment, should determine priority. If services for severe illnesses are very costly the cost should be shared across the whole community	2.28	1.04	0.05	23.6	41.0	22.2	10.0	3.2
Illnesses which are very expensive to treat should not be covered by Medicare	4.01	1.03	0.05	3.2	6.3	13.9	39.6	37.0

Appendix 6 Orphan Drug Avatar Script

Orphan Drugs Script: Sort 1

SCREEN	VISION	AVATAR AUDIO
o1as12 Welcome	Avatar 1024x576 with subtitles Avatar 1024x576 WITHOUT subtitles from now on	Hello and welcome. I'll be your guide today. It's really important that you do this survey on a computer with a screen that is larger than an iPad and it's also important that you can hear my voice so please plug in your headphones or turn up your speakers. If you can't enable sound on the computer you're on, please return to this survey when you're on a computer with sound capabilities. Thanks for participating in this project. It is part of a research program at the Centre for Health Economics at Monash University and it is not connected with any political or commercial interests. Your answers will help us understand how the public thinks Medicare should allocate its budget between various patients **when the cost of treating them varies significantly and it is not enough to give a complete cure to everyone.** So I will be asking you to evaluate and compare different health conditions. If you ever need to pause one of these videos just hover over it and you'll see a pause button. If you want to hear a video again, when it's finished, click on it and it will replay. . If you are willing to continue now please click next below.
01bs12 gender age	Avatar 400x300	During this survey you will remain anonymous but we do need your age and gender so please enter those below then click next to read the participant information.
01cs12	Please read the participant information below then click next to participate in this survey.	NO AVATAR
01ds12	Avatar 400x300	Thanks. Now please read the descriptions of the 4 health states. They describe various levels of problems with walking, and self care needs such as washing, dressing and toileting oneself. Write '1' next to the description you think is the best health state, '2' next to the second best, '3' next to 3rd best and '4' next to what you think is the worst health state.
o2s1 Introduce Rating Scale Rate Own Health	Avatar 400x300	Good. Thanks. Now I'd like you to use the health rating scale on screen to indicate how you would rate your present health. This scale ranges from 0 (or death) at the bottom of the scale, all the way up to 100 at the top which represents best possible health. It's not perfect health, but it's like when you say "I'm feeling 100% today".

		Where do you feel you are on the scale when taking into account both your physical and mental health? Please write your score below then click next.
o3s12 Introduce Rating Scale Rate 4 Health States	Avatar 400x300	Now I'd like you to use the same scale to rate the 4 health states below which all involve problems with walking and self care. Self care is your ability to look after your own personal needs including washing, dressing and toileting. First please imagine how you would feel if you were in each health state then rate each of them from 0-100. Remember, 0 represents death and 100 represents the best health possible. Slight problems with walking and self care are better than severe problems so that health state would naturally be higher up the scale. 'Unable to walk and self care' is the worst of the 4 health states and should therefore rate the lowest. When you're ready, please imagine yourself in each of these health states and give them a rating.
o4s12 Introduce Rating Scale Australian (VAS) Norms	Avatar 1024x576 Animation	Thanks. Those four health states will be used later. Here are the average scores we obtained in an earlier survey. Where zero represents death and 100 represents the best possible health with NO problems, the Australian community has rated slight problems with walking and self care at 95, moderate problems at 80, severe problems at 55, and extreme problems where one is unable to walk and self care at 30. So we're going to work with these ratings for the remainder of this survey. (continue as one video since there's no interaction)
o4 (cont) Introduce % Cost Spent	Avatar 1024x576 Animation	Now suppose there is a disease which will make people die if they are untreated. A treatment that completely cures <u>one</u> patient costs $1,000. If a patient gets any treatment at all they'll be saved but with anything less than the *complete* treatment they will be left with problems with walking and self care. The effect of any treatment lasts for 10 years; after that time the treatment needs to be repeated. The severity of the problems depends upon how much is spent on the treatment. To keep things really simple we assume that <u>**the percentage of the full cost of treatment that is spent on them, creates the same percentage of health.**</u> For example, here the full cost of treatment is $1000. If $800 is spent which is 80% of the full cost, then the patient will be restored to 80% health (continue as one video)
o4 cont Introduce 2 People	Animation continues...	Suppose now that two people, Patient X and Patient Y have this disease. With some treatment their lives are saved but they are left with problems with walking and self care, as shown. Remember, a complete cure costs $1000 and lasts 10 years. Patient X has received $200 which is 20% of the cost of a complete cure, and consequently has reached only 20% of best possible health and would need another $800 to be completely cured.

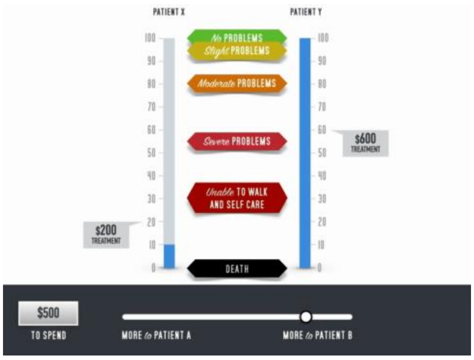
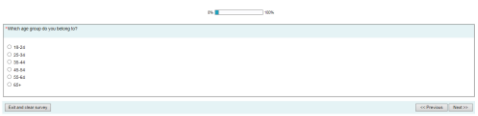
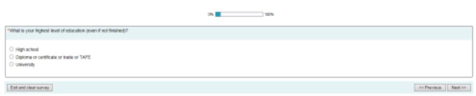
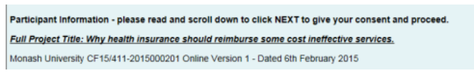
		Patient Y has received $600 which is 60% of the cost of a complete cure, and consequently has achieved 60% of best possible health and would need another $400 to be completely cured. Suppose the government has given $500 more to spend on the 2 patients. As this is not enough to cure them both how would you divide it between X and Y? Now we know most people don't like maths so to keep this really simple, I've added more blue shading to represent the extra $500 and you can use the slider to decide how to share this money between the patients. . As you move the handle on the slider, the $500 is divided accordingly between patient X and patient Y. Click next then on the next screen you can move the slider and submit your answer.
o5s12	Question with slider that divides $500 between two patients.	**NO VIDEO**
o6s1 Town with 2 illnesses	Avatar 1024x576 Animation Avatar 1024x576	Great. Now we come to the main questions. Suppose you live in a small town of 1,000 people. Experts advise that next year two illnesses will occur which we will call illness A and illness B. Both illnesses have the same symptoms which are problems with mobility and self care. 5 people will get illness A and 100 people will get illness B. Anyone in the town – men and women of all ages – may get one of the illnesses. You, personally, could be one of these people. As with the previous examples: both illnesses are life threatening and **without treatment the patients will die**, With some treatment the patients will be saved from death but left with problems with walking and self care such as dressing washing and toileting The more treatment a patient receives, the more their health will improve until the illness is cured and the effect of any treatment, whether it is a partial improvement or full cure lasts 10 years after which it will have to be repeated. But here's the catch... Because the causes of the illnesses are very different the cost of treatment for the two illnesses is very different. A full cure for each person with illness B costs $1,000 whereas a full cure for each person with illness A costs $20,000. That's 20 times as much! The government has allocated a fixed fund to treat the two illnesses. But the amount in all the questions that follow is not enough to provide a full cure for everyone. Extra private spending is not possible and no other services will cure the illnesses. The questions are about how you think the government should distribute its money. In the first 5 questions the fixed government fund is $100,000 and using the slider you can choose how to divide the money between the 2 illnesses and therefore how much health to create for each group of patients. Notice I'm talking about the amount of health created which is represented by the size of the blue areas.

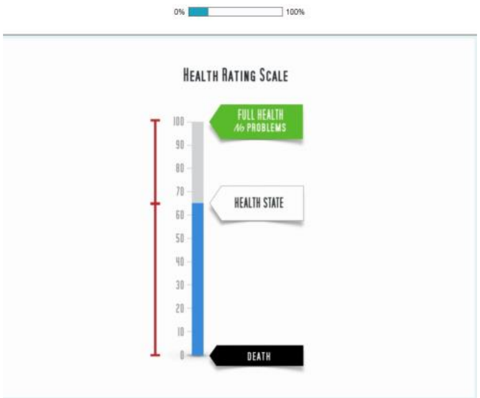
	Slider drawer comes up and fund amount	Moving the handle all the way to right creates the maximum total amount of health as shown by the large blue area. This is because Illness B is cheaper to cure so you can buy the most units of health if you spend all of the fund on Illness B. But if you do this, the 5 patients with Illness A get nothing and they all die. Moving the handle all the way to left creates the minimum total amount of health as shown by the tiny blue area. This is because Illness A is more expensive to cure so you're buying the least units of health if you spend all of the fund on Illness A. And if you do this, the 100 patients with Illness B get nothing and they all die. (continue video)
	Animation continues...	You'd probably think that's a bad idea and may not agree with the distribution. So if you slide the handle all the way to the right you're creating the maximum amount of health by giving everything to B, but should the 5 people with illness A die just because their treatment is more expensive? Or do you want to save their lives too and share a smaller total amount of health between A and B. And if you share, how much health do you want to give to A? (continue video)
	Animation continues...	For instance you could allocate half ($50,000) to each group so no-one dies, but both groups of patients would be left with severe problems with walking and self care. And it also means that the total health created with the fund is less because there has been a big reduction in the blue area in B with only a small increase in the blue area in A. So this illustrates the problem we're asking you: Do you maximise total health which means give everything to B and let patients with illness A die or do you reduce total health and share it with A.
	Animation continues... The slider moves to a few different positions so the user gets the idea that they can choose any amount.	(continue video)
	Animation continues… Avatar 1024x576	In the following questions **I would like you to position the handle to** show your opinion of the best outcome: how you think the government should distribute the money. You will answer a total of 15 questions where the number of patients and cost of treatment vary. The decisions are quite hard. The choice is always between more health for more people on the one hand, and on the other hand, less health overall but sharing it with high cost patients. Press next to start the first set of 5 questions.

o7s12 Set 1 5xA 100xB	Avatar 400x300 (or audio only) (Question 1 is the same as in the prior examples ie FULL treatment for illness B costs $1000 per person and FULL treatment for illness A costs $20,000 per person.)	Your time has come. Please move the handle to show how much health you'd choose to create with the government funds then submit your answer below.
o8s12 A=$15,000	Avatar 400x300 (or audio only)	**Question 2:** Great. That's the first of the 15 questions. Now thanks to some medical advancements, the full cost of treating a patient with illness A has fallen from $20,000 to $15,000. A full treatment for illness B still costs $1000. Remember to imagine what it would be like for someone to have the illnesses then move the handle to show how you would divide health between A and B.
o9s12 A=$10,000	Avatar 400x300 (or audio only)	**Question 3: (replayed later)** The full cost of treating a patient with Illness A has fallen to $10,000; B still costs $1000. How would you divide health between A and B now?
o10s12 A=$5,000	Avatar 400x300 (or audio only)	**Question 4: (replayed later)** The full cost of treating a patient with illness A is now $5000; B still costs $1000. Please move the handle to divide health between the two groups.
o11s12 A=$2,000	Avatar 400x300 (or audio only)	**Question 5: (replayed later)** Okay last one in this set of questions. The full cost of treating a patient with illness A is now just $2000; B remains $1000. Remember to think of what it would be like for someone to have the illnesses then move the handle to show how you would divide health now.
o12s1 Set 2 5xA 300xB	Animation	Remember our small town of 1000 people? Well now 300 people have illness B but fortunately the government has also tripled the fund. As a result you'll notice that creating health for people with Illness A doesn't impact people with Illness B quite so much. The next 5 questions are in the same style as the last 5. Let's get started.
o13s12 A=20000	Avatar 400x300 (or audio only)	**Question 1: (replayed later)** Please move the handle to show how much health you'd choose to create with the government funds.
o14s12 A=15000	Avatar 400x300 (or audio only)	**Questions 2:** Again thanks to some medical advancements, the full cost of treating a patient with illness A has fallen from $20,000 to $15,000. A full treatment for illness B still costs $1000.
o15s12 **(replay o9)** A=10000	Avatar 400x300 (or audio only)	**Question 3: (replay o9?)** The full cost of treating a patient with Illness A has fallen to $10,000; B still costs $1000. How would you divide health between A and B now?

o16s12 **(replay o10)** A=5000	Avatar 400x300 (or audio only)	**Question 4: (replay o10?)** The full cost of treating a patient with illness A is now $5000; B remains $1000. Move the handle according to how you'd divide health between the two.
o17s12 **(replay o11)** A=2000	Avatar 400x300 (or audio only)	**Question 5: (replay o11?)** Okay last one in this set of questions. The full cost of treating a patient with illness A is now just $2000; B remains $1000. Remember to think of what it would be like for someone to have the illnesses. Move the handle to show how you would divide health now.
o18s1 Set 3 5xA 600xB	Animation 1024x768.	Well done. Only 5 questions left. Now 600 people have illness B so the government has doubled its fund. Creating health for people with Illness A now has even less impact on people with Illness B. The final 5 questions are in the same style so let's get started.
o19s12 A=20000	Avatar 400x300 (or audio only)	**Question 1: (replay o13)** Please move the handle to show how much health you'd choose to create with the government funds.
o20s12 **(replay o14)** A=15000	Avatar 400x300 (or audio only)	**Questions 2: (replay o14)** Again, thanks to some medical advancements, the full cost of treating a patient with illness A has fallen from $20,000 to $15,000. a full treatment for illness B still costs $1000.
o21s12 **(replay o9)** A=10000	Avatar 400x300 (or audio only)	**Question 3: (replay o9?)** The full cost of treating a patient with Illness A has fallen to $10,000; B still costs $1000. How would you divide health between A and B now?
o22s12 **(replay o10)** A=5000	Avatar 400x300 (or audio only)	**Question 4: (replay o10?)** The full cost of treating a patient with illness A is now $5000; B remains $1000. Move the handle according to how you'd divide health between the two.
o23s12 A=2000	Avatar 400x300 (or audio only)	**Question 5:** And now for the last question. The full cost of treating a patient with illness A is now just $2000; B remains $1000. Remember to think of what it would be like for someone to have the illnesses then move the handle to show how you would divide health now.
o24s12	Avatar 400x300	Thanks so much for carefully considering all the questions in this survey. We appreciate your answers and your time. Make it a great day.

Orphan Drugs Script: Sort 2

SCREEN	VISION	AVATAR AUDIO
o1as12 Welcome	Avatar 1024x576 with subtitles  Avatar 1024x576 WITHOUT subtitles from now on 	Hello and welcome. I'll be your guide today. It's really important that you do this survey on a computer with a screen that is larger than an iPad and it's also important that you can hear my voice so please plug in your headphones or turn up your speakers. If you can't enable sound on the computer you're on, please return to this survey when you're on a computer with sound capabilities. Thanks for participating in this project. It is part of a research program at the Centre for Health Economics at Monash University and it is not connected with any political or commercial interests. Your answers will help us understand how the public thinks Medicare should allocate its budget between various patients **when the cost of treating them varies significantly and it is not enough to give a complete cure to everyone.** So I will be asking you to evaluate and compare different health conditions. If you ever need to pause one of these videos just hover over it and you'll see a pause button. If you want to hear a video again, when it's finished, click on it and it will replay. . If you are willing to continue now please click next below.
		
		
		
		
		
01ds2	Avatar 400x300	Thanks. Now please read the descriptions of the 4 health states below and number them 1 through 4 with 1 being

		what you think is the best health state and 4 being what you think is the worst.
o1es2		Thanks for that. In a moment I'll ask you to evaluate some health states that will be used in later questions. We'll use a rating scale like this one. The scale lets you show how strongly you feel about the health states we'll describe to you. On the scale, 0 represents death or a health state as bad as death, and 100 represents full health. On our scale 'Full Health' does not mean perfect health but 'no problems with moving about by yourself and no problems with self care: so it's good, but not perfect health'. I'll ask you to imagine what it would be like to be in some hypothetical health states, and where you would place them on the scale. There are no right or wrong answers. The questions are about your feelings; the better you feel a health state is, the higher the score you should give it. If you were to give a Health State a rating of 65, then this means you feel the Health State is about twice as far from death as it is from full health. Click next to continue.
o3s2 Introduce Rating Scale Rate 4 Health States	Avatar 400x300 	In a moment I'd like you to rate the 4 health states on screen which are about walking and self care. Self care is your ability to look after your own personal needs including washing, dressing and toileting. Most importantly, you should imagine what it would be like if you were in the health states described. We want to know how strongly you would feel about them. So on a scale of 0-100, how would you rate these 4 health states?
o4s2 Introduce Rating Scale Australian (VAS) Norms	Avatar 1024x576	Thanks. Those four health states will be used later. Here are the average scores we obtained in an earlier survey. Where zero represents death and 100 represents full health with NO problems, the Australian community has rated slight problems with walking and self care at 95, moderate problems at 80, severe problems at 55, and extreme problems where one is unable to walk and self

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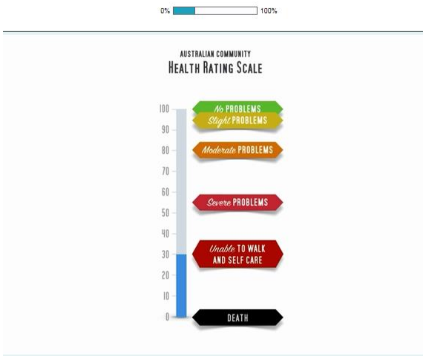
<td>
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<td>care at 30. So we’re going to work with these ratings for the remainder of this survey.

(continue as one video since there’s no interaction)</td>
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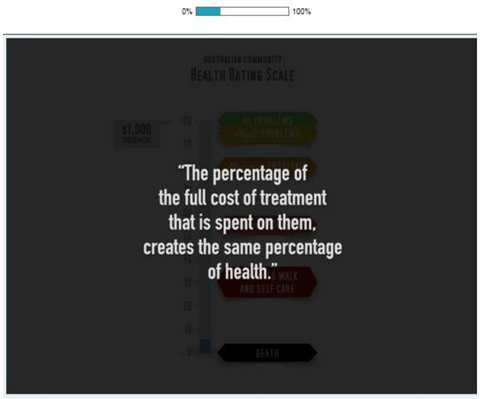
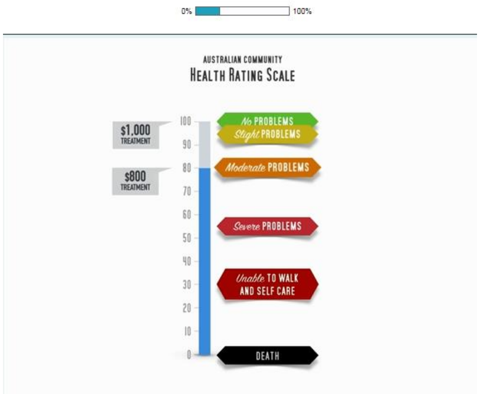
<td>o4 (cont)
Introduce
% Cost
Spent</td>
<td>



Avatar 1024x576</td>
<td>Now suppose there is a disease which will make people die if they are untreated.

A treatment that completely cures <u>one</u> patient costs $1,000.
If a patient gets any treatment at all they’ll be saved but with anything less than the complete treatment they will be left with problems with walking and self care.
The effect of any treatment lasts for 10 years; after that time the treatment needs to be repeated.

The severity of the problems depends upon how much is spent on the treatment. To keep things really simple we assume that <u>the percentage of the full cost of treatment that is spent on them, creates the same percentage of health.</u>

For example, here the full cost of treatment is $1000. If $800 is spent which is 80% of the full cost, then the patient will be restored to 80% health.

(continue as one video)</td>
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<td>o4 cont
Introduce 2
People</td>
<td>Animation continues...

Avatar 1024x576</td>
<td>Suppose now that two people, Patient X and Patient Y have this disease. With some treatment their lives are saved but they are left with problems with walking and self care, as shown.
Remember, a complete cure costs $1000 and lasts 10 years.
Patient X has received $200 which is 20% of the cost of a complete cure, and consequently has reached only 20% of best possible health and would need another $800 to be completely cured.</td>
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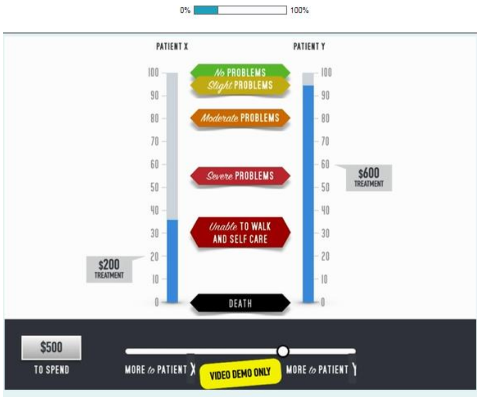
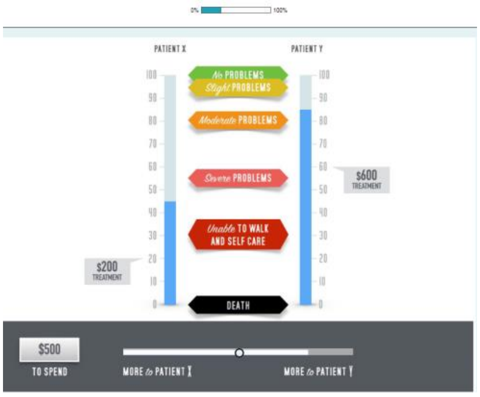
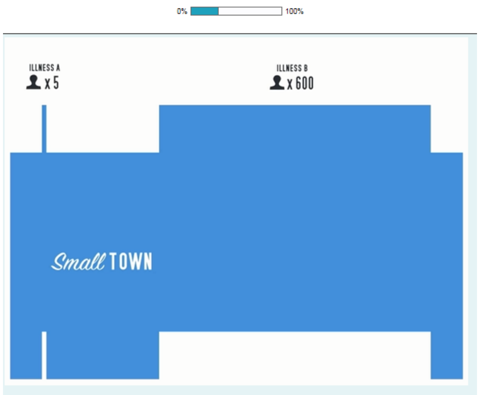
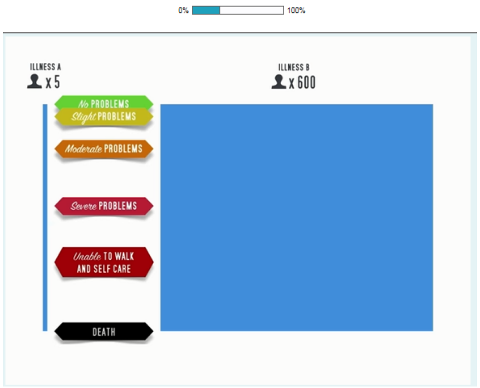
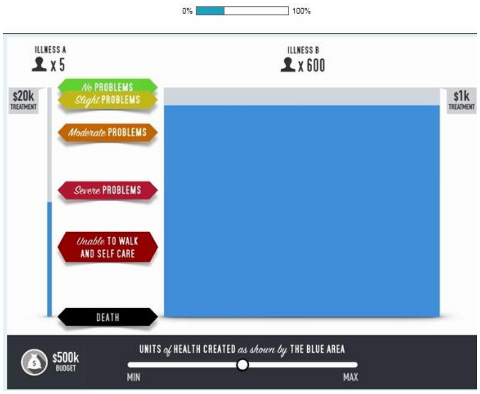
		Patient Y has received $600 which is 60% of the cost of a complete cure, and consequently has achieved 60% of best possible health and would need another $400 to be completely cured. Suppose the government has given $500 more to spend on the 2 patients. As this is not enough to cure them both how would you divide it between X and Y? Now we know most people don’t like maths so to keep this really simple, I’ve added more blue shading to represent the extra $500 and you can use the slider to decide how to share this money between the patients. . As you move the handle on the slider, the $500 is divided accordingly between patient X and patient Y. Click next then on the next screen you can move the slider and submit your answer.
o5s12		**NO VIDEO (**Question with slider that divides $500 between two patients.)
o6s2 Town with 2 illnesses	Avatar 1024x576 	Great. Now we come to the main questions. Suppose you live in a small town. Experts advise that next year two illnesses will occur which we will call illness A and illness B. Both illnesses have the same symptoms which are problems with mobility and self care. 5 people will get illness A and 600 people will get illness B. Anyone in the town – men and women of all ages – may get one of the illnesses. You, personally, could be one of these people. Both illnesses are life threatening and **without treatment the patients will die**. With some treatment the patients will be saved from death but left with problems with walking and self care such as dressing washing and toileting. The more treatment a patient receives, the more their health will improve until the illness is cured and the effect of any treatment, whether it is a partial improvement or full cure lasts 10 years after which it will have to be repeated. But here’s the catch... Because the causes of the illnesses are very different the costs of treatment for the two illnesses are very different. A full cure for each person with illness B costs $1,000





whereas a full cure for each person with illness A costs $20,000. That's 20 times as much!

The government has allocated a fixed budget to treat the two illnesses. But the amount in all the questions that follow is not enough to provide a full cure for everyone. Extra private spending is not possible and no other services will cure the illnesses.

The questions are about how you think the government should distribute its money. In the first 5 questions the fixed government budget is $600,000 and using the slider you can choose how to divide the money between the 2 illnesses and therefore how much health to create for each group of patients.

Notice I'm talking about the amount of health created which is represented by the size of the blue areas.

Moving the handle all the way to right creates the maximum total amount of health. This is because Illness B is cheaper to cure so you can buy the most units of health if you spend all of the fund on Illness B. But if you do this, the 5 patients with Illness A get nothing and they all die.

Moving the handle all the way to left creates the minimum total amount of health. This is because Illness A is more expensive to cure so you're buying less units of health when you spend on Illness A. When you do this, the 600 patients with Illness B get less health. You can see this because the total blue area in both A and b combined is much smaller than before.

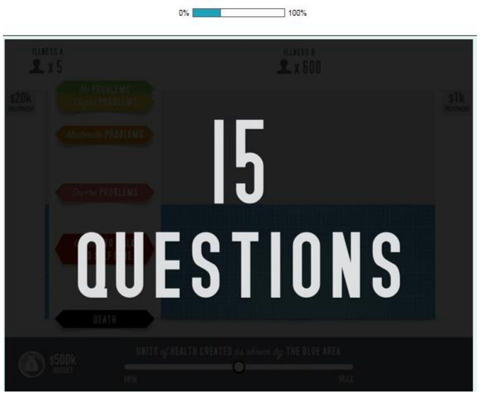
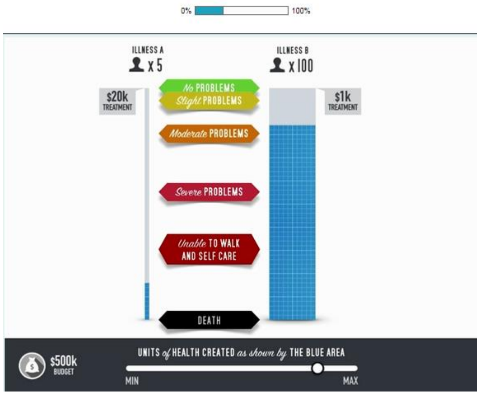
So if you slide the handle all the way to the right you're creating the maximum amount of health by giving everything to B, but should the 5 people with illness A die just because their treatment is more expensive? Or do you want to save their lives too and share a smaller total amount of health between A and B. And if you share, how much health do you want to give to A?

In all of the following questions **I would like you to position the handle to** show your opinion of the best outcome: how you think the government should distribute the money.

And you don't have to worry about adding up the amount you give to the patients - you'll be spending the full budget wherever you position the handle on the slider.

You will answer a total of 15 questions where the number of patients and cost of treatment vary. The decisions are quite hard.

When there are less patients with illness B you'll notice that they are even more impacted when creating health for people with Illness A. For a small increase in A there is a large decrease in B.

	 	Regardless, the choice is always between more health for more people on the one hand, and on the other hand, less health overall but sharing it with high cost patients. Press next to start the first set of 5 questions.
o11as2 Set 1 5xA 600xB	Avatar 400x300 (or audio only) 	**Question 1:** Your time has come. Please move the handle to show how you would divide the budget, how much health you would create and how you would share it around.

o11bs2	 NEW	**Question 2: Now thanks to some medical advancements,** the full cost of treating a patient with illness A has fallen from $20,000 to $15,000. A full treatment for illness B still costs $1000. As a result you will notice that when you change the amount of health you give to A it will have less effect on B. Remember to imagine what it would be like for someone to have the illnesses then move the handle to show how you would divide health between A and B.
o11cs2 **(replay o9)**		Question 3: **(replay o9)** The full cost of treating a patient with Illness A has fallen to $10,000; B still costs $1000. How would you divide health between A and B now?
o11ds2 **(replay o10)**		Question 4: **(replay o10)** The full cost of treating a patient with illness A is now $5000; B still costs $1000. Please move the handle to divide health between the two groups.

o11es2 **(replay o11)**		Question 5: **(replay o11)** The full cost of treating a patient with illness A is now just $2000; B remains $1000. Remember to think of what it would be like for someone to have the illnesses then move the handle to show how you would divide health now.
o12s2		Remember our small town? Well now experts predict that only 300 people will have illness B and so the government has also cut the budget by half to $250,000. As a result you'll notice that creating health for people with Illness A makes a bigger difference for people with Illness B. The cost of curing illness A has returned to $20,000. Remember, the illness could affect anyone in the town, including you. The next 5 questions are in the same style as the last 5. Click next and let's get started.
o13s2 A=20000	Avatar 400x300 (or audio only) 	**Question 1: (replayed later)** Please move the handle to show how much health you'd choose to create with the government budget.
o14s12 A=15000	Avatar 400x300 (or audio only)	**Questions 2:** Again thanks to some medical advancements, the full cost of treating a patient with illness A has fallen from $20,000 to $15,000. A full treatment for illness B still costs $1000.

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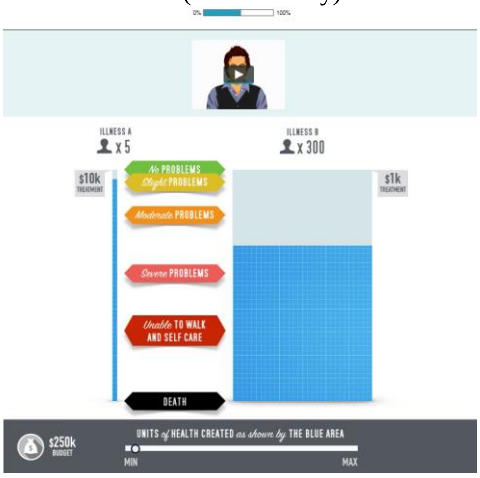
(replay o9)
A=10000</td>
<td>Avatar 400x300 (or audio only)

</td>
<td>Question 3: (replay o9?) The full cost of treating a patient with Illness A has fallen to $10,000; B still costs $1000. How would you divide health between A and B now?</td>
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<tr>
<td>o16s12
(replay o10)
A=5000</td>
<td>Avatar 400x300 (or audio only)

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<td>Question 4: (replay o10?) The full cost of treating a patient with illness A is now $5000; B remains $1000. Move the handle according to how you’d divide health between the two.</td>
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<tr>
<td>o17s2
(replay o11)
A=2000</td>
<td>Avatar 400x300 (or audio only)</td>
<td>Question 5: Okay last one in this set of questions. The full cost of treating a patient with illness A is now just $2000; the cost of curing B remains $1000. Remember to think of what it would be like for someone to have the illnesses. Move the handle to show how you would divide health now.</td>
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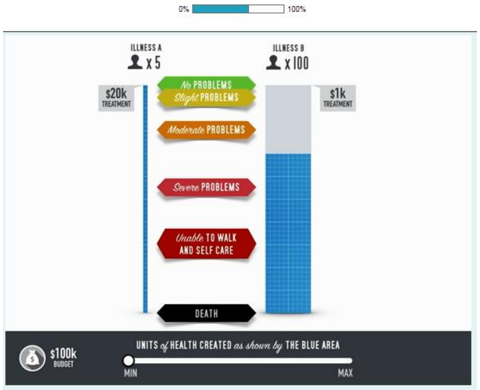
o18s2	Animation 1024x768 	Well done. Only 5 questions left. Now only 100 people have illness B so the government has reduced its budget even further to $80,000. Creating health for people with Illness A now has much more impact on people with Illness B. The cost of curing illness b has returned to $20,000. The final 5 questions are in the same style so click next and let's get started.
o19s12 A=20000	Avatar 400x300 (or audio only) 	**Question 1: (replay o13)** Please move the handle to show how much health you'd choose to create with the government funds.
o20s12 **(replay o14)** A=15000	Avatar 400x300 (or audio only)	**Questions 2: (replay o14)** Again, thanks to some medical advancements, the full cost of treating a patient with illness A has fallen from $20,000 to $15,000. a full treatment for illness B still costs $1000.

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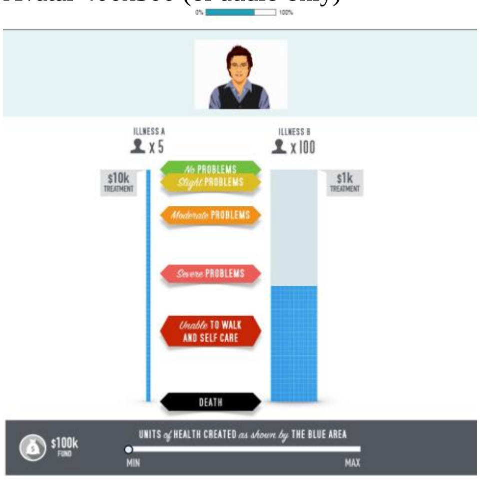
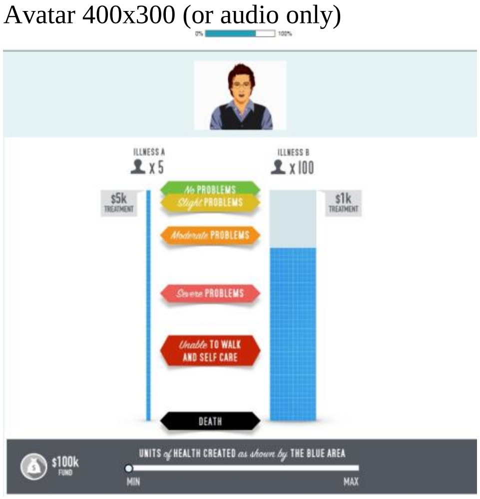
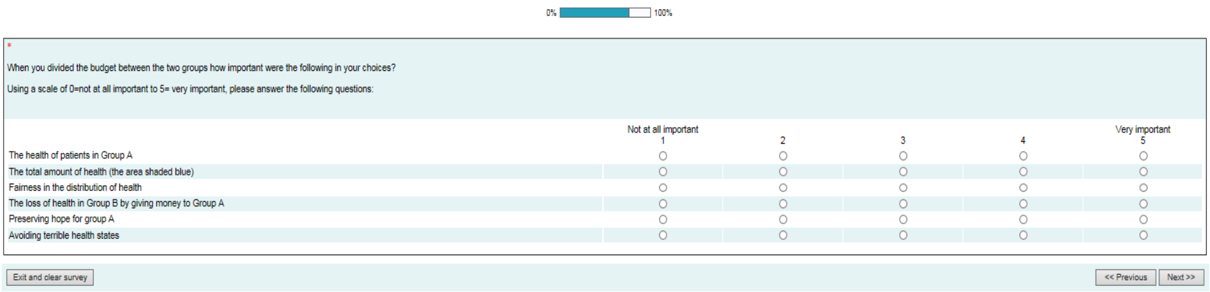
(replay o9)
A=10000</td><td>Avatar 400x300 (or audio only)

</td><td>Question 3: (replay o9?) The full cost of treating a patient with Illness A has fallen to $10,000; B still costs $1000. How would you divide health between A and B now?</td></tr>
<tr><td>o22s12
(replay o10)
A=5000</td><td>Avatar 400x300 (or audio only)

</td><td>Question 4: (replay o10?) The full cost of treating a patient with illness A is now $5000; B remains $1000. Move the handle according to how you'd divide health between the two.</td></tr>
<tr><td>o23s12
A=2000</td><td>Avatar 400x300 (or audio only)</td><td>Question 5: And now for the last question. The full cost of treating a patient with illness A is now just $2000; B remains $1000. Remember to think of what it would be like for someone to have the illnesses then move the handle to show how you would divide health now.</td></tr>
</table>

		
o24s12	Avatar 400x300	Thanks so much for carefully considering all the questions in this survey. We appreciate your answers and your time. Make it a great day.



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*

When you divided the budget between the two groups how important were the following in your choices?

Using a scale of 0=not at all important to 5= very important, please answer the following questions:

	Not at all important 1	2	3	4	Very important 5
The health of patients in Group A	○	○	○	○	○
The total amount of health (the area shaded blue)	○	○	○	○	○
Fairness in the distribution of health	○	○	○	○	○
The loss of health in Group B by giving money to Group A	○	○	○	○	○
Preserving hope for group A	○	○	○	○	○
Avoiding terrible health states	○	○	○	○	○

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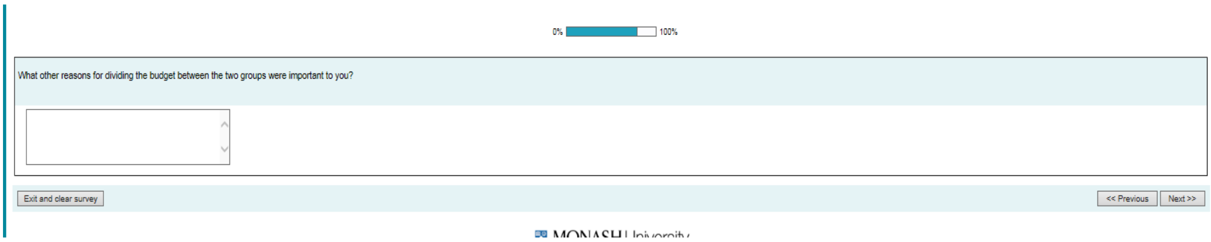

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What other reasons for dividing the budget between the two groups were important to you?

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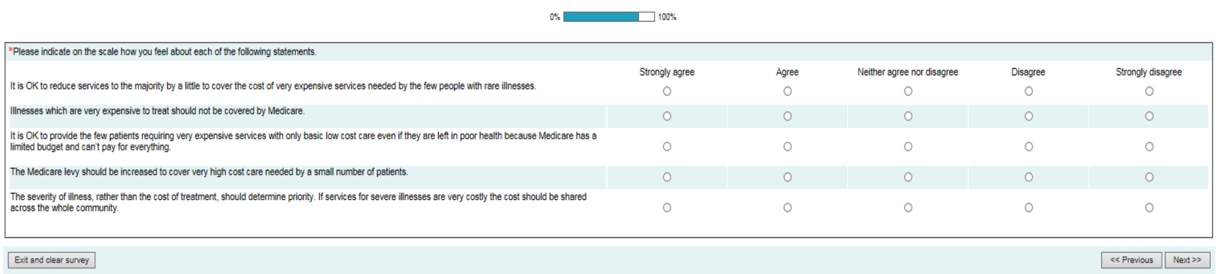

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*Please indicate on the scale how you feel about each of the following statements.

	Strongly agree	Agree	Neither agree nor disagree	Disagree	Strongly disagree
It is OK to reduce services to the majority by a little to cover the cost of very expensive services needed by the few people with rare illnesses.	○	○	○	○	○
Illnesses which are very expensive to treat should not be covered by Medicare.	○	○	○	○	○
It is OK to provide the few patients requiring very expensive services with only basic low cost care even if they are left in poor health because Medicare has a limited budget and can't pay for everything.	○	○	○	○	○
The Medicare levy should be increased to cover very high cost care needed by a small number of patients.	○	○	○	○	○
The severity of illness, rather than the cost of treatment, should determine priority. If services for severe illnesses are very costly the cost should be shared across the whole community.	○	○	○	○	○

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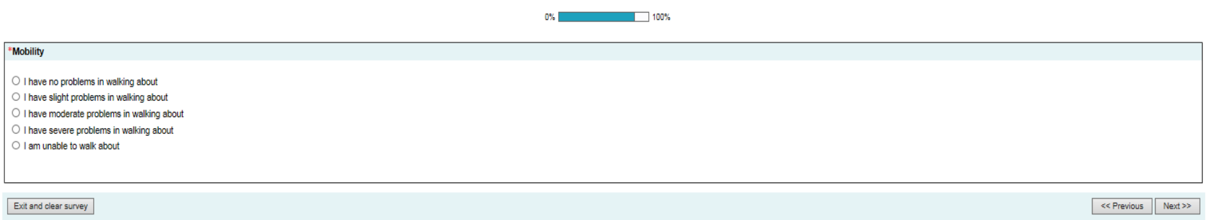

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***Mobility**

○ I have no problems in walking about
○ I have slight problems in walking about
○ I have moderate problems in walking about
○ I have severe problems in walking about
○ I am unable to walk about

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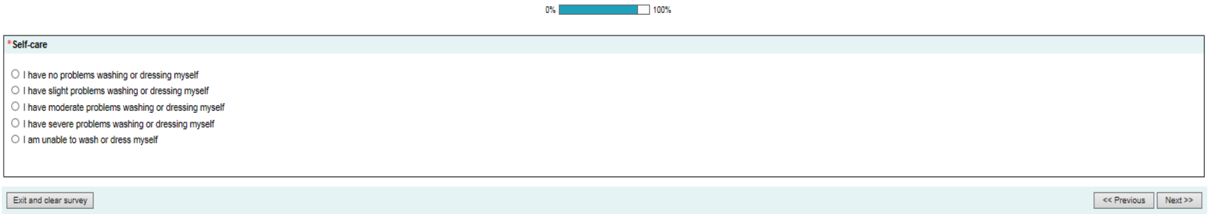
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*Self-care

- I have no problems washing or dressing myself
- I have slight problems washing or dressing myself
- I have moderate problems washing or dressing myself
- I have severe problems washing or dressing myself
- I am unable to wash or dress myself

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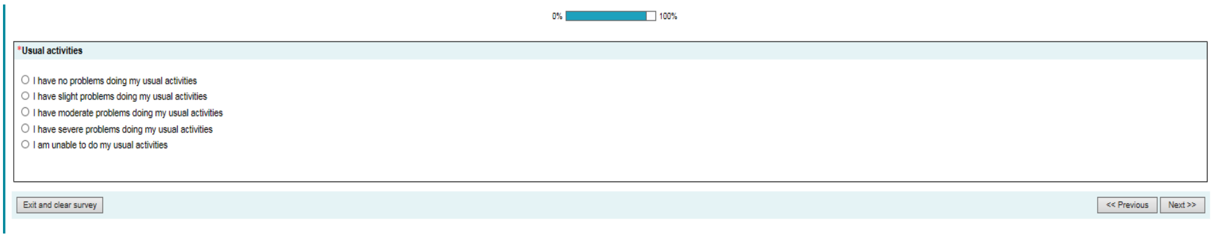
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*Usual activities

- I have no problems doing my usual activities
- I have slight problems doing my usual activities
- I have moderate problems doing my usual activities
- I have severe problems doing my usual activities
- I am unable to do my usual activities

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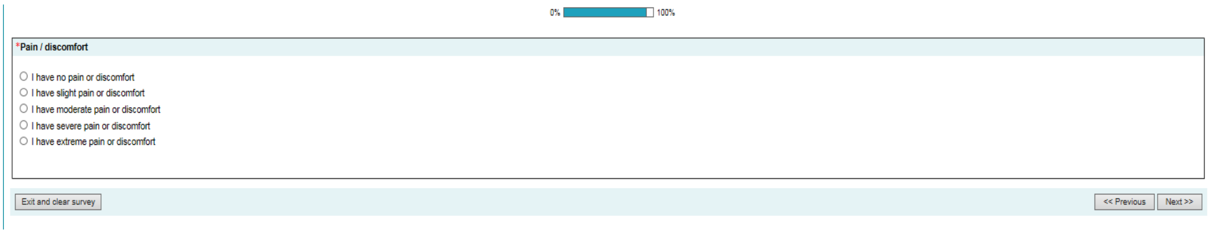
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*Pain / discomfort

- I have no pain or discomfort
- I have slight pain or discomfort
- I have moderate pain or discomfort
- I have severe pain or discomfort
- I have extreme pain or discomfort

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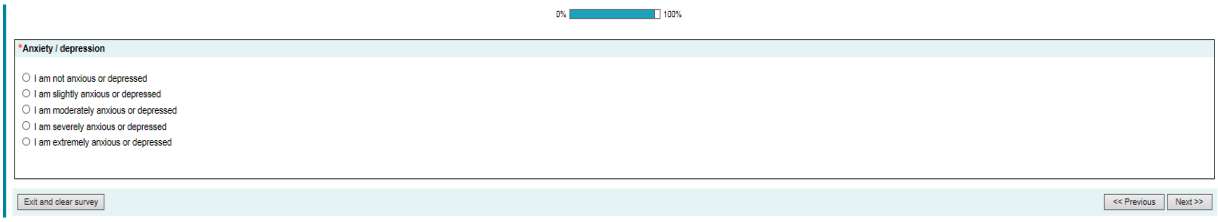
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*Anxiety / depression

- I am not anxious or depressed
- I am slightly anxious or depressed
- I am moderately anxious or depressed
- I am severely anxious or depressed
- I am extremely anxious or depressed

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