

UTILITIES FOR INFUSION THERAPY IN TYPE 1 DIABETES

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Abstract

Purpose:

Recent advances in monoclonal antibody therapies offer the prospect of the prevention or amelioration of type 1 diabetes (T1DM). The present study was designed to capture UK (English and Scottish) preference weights for the process of undergoing infusion therapy and the likely outcomes of treatment for children (8-12yrs); adolescents (13-17yrs) and adults. Such values could be used in a cost effectiveness analysis.

Methods:

In depth interviews were conducted with adults (n=4), and parents of children (n=4), and adolescents (n=4) with type 1 diabetes to characterise their quality of life. These data, an interview with a clinical expert and findings from a literature review were used to construct vignette descriptions of health. Vignettes described T1DM; T1DM with reduced need for insulin; T1DM insulin free; receiving infusion therapy and receiving infusion therapy plus acute phase reaction. Separate vignettes described adults, adolescents and children. Following expert review of content validity vignettes were rated (using the standard gamble (SG) interview) by the general public, adult patients with diabetes and parents of children with diabetes. Participants also completed other outcome measures – EQ-5D, PedsQL, Hypoglycemia Fear Survey (data will be reported). Mixed model analyses were used to estimate the influence on utility of different participant characteristics.

Results:

The mixed model analysis indicated that each health state was a significant predictor of utility and the T1DM participants gave significantly different ratings to general public (P=0.02).

Conclusion:

The general public and people with diabetes (or parents of children) all place significant value on reducing the need for insulin injections, and all recognise the disutility of undergoing infusion based therapies. Compared to T1DM parents and patients the general public indicated that the infusion therapy would have a greater negative impact.

Introduction

- Type 1 diabetes mellitus (T1DM) leaves people unable to produce insulin. As a result people are left to manage the disease through a combination of a carefully calculated diet, planned physical activity, repeated blood glucose testing and multiple daily insulin injections
- T1DM has a significant impact on health related quality of life (HRQL) of patients in terms of both the management of the disease and the impact of complications
- Improvements (or avoidance of deterioration) in HRQL are clearly of value to patients and such information can be used to communicate the benefits of an intervention to help guide patient decision making
- In addition, improvements in HRQL can be used as a measure of benefit in economic evaluations. HRQL data have become a key component of economic evaluations and are a stated requirement of reimbursement bodies such as the UK's National Institute for Health and Clinical Excellence (NICE)
- HRQL data are used to weight survival in the estimation of quality adjusted life years (QALYs) which are the most important measure of benefit in economic evaluation for NICE and other bodies
- HRQL data have to be weighted by people's views or preferences (either society or patients) regarding how severe they are in order for them to be suitable for estimating QALYs

Objective

- The treatment of T1DM with an infusion based therapy has not previously been explored in terms of its impact on HRQL. This study was designed to capture utilities based on the preferences of the society, adults with T1DM and parents of children and young adults with T1DM.

Methods

- Development of the health states for use in the utility elicitation phase was achieved through a number of complimentary processes: a review of the published literature, patient-focused qualitative work, and interviews with healthcare professionals.
- Literature Review:** A review was conducted of the relevant academic literature to examine the HRQL impact of T1DM for adult, adolescent and child patients
- The literature search was conducted using Embase and restricting articles to those published in the English language and between the years 2000 and 2008
- Embase and PubMed were searched (terms = Insulin dependant diabetes mellitus AND quality of life) to identify articles which were informative regarding the nature of the impact of T1DM on HRQL
- From the search 604 abstracts were reviewed and 28 were identified for full-review
- Patient Focused Qualitative Work:**
 - Twelve in depth face-to-face interviews were conducted with individuals who had direct experience of dealing with the condition (4 adults with diabetes, 4 parents of adolescents with diabetes aged 13-17 and 4 parents of children with diabetes aged 8-12)
 - A thematic analysis was conducted using the qualitative analysis software tool, ATLAS.ti. The analysis was designed to identify themes and explore the possible inter-relationships that may exist between them
- Interviews with Healthcare Professionals:**
 - A consultant endocrinologist, a diabetologist, and a diabetes specialist nurse were interviewed, all of whom had extensive experience of dealing with patients with T1DM
 - The experts were encouraged to provide their perspective on the likely impact of T1DM for patients of varying ages and to relay their experiences of clinical practice with the patient group
- Health State Development and Pilot Study**
 - The first draft of the health state descriptions were produced based upon the interviews and literature review
 - The health states described different areas of functioning (specifically physical, psychological and social functioning), symptoms, usual activities and work and were designed to cover the different main elements of HRQL
 - Interviews with clinicians, patients and parents were conducted to assess the content validity of the states
 - A pilot interviewing phase was conducted with 12 members of the general public.
 - 22 different health states were used in the study -with each health state tailored to the specific population of interest

Example Health State

Adult Type 1 Diabetes on Infusion Therapy with an Acute Phase Reaction

- You have an illness that requires you to inject yourself 2-5 times a day and check your blood sugar levels. Your illness could lead to a range of different health problems, including problems with your eyesight, which could greatly affect your quality of life.
- You attend clinic everyday for 3-4 hours which includes an intravenous treatment lasting 45 minutes. The treatment sometimes causes a rash on your arm.
- You are able to do light exercise and have no problems with moving or walking about. Your illness often interferes with seeing your friends and family. You must carry a small medical kit with you at all times.
- You are not able to go to work or do all of your usual day to day activities.
- You are able to wash, dress and care for your self normally. You have to be very careful what and when you eat and drink especially if you are away from home.
- You experience some pain from your injections. You spend a lot of time worrying about your health getting worse and you sometimes feel low or depressed.

Statistical Analyses

- Statistical Analyses
 - All analyses were described in an *a priori statistical analysis plan*
 - The standard gamble data were rescaled against worst health so that all utilities were on a 0 to 1.0 scale
 - Mixed models were fitted using SAS

Results

- All interviews were conducted by trained interviewers
- A total of 100 members of the general public were recruited 51 adults with T1DM (36 in England, 15 in Scotland) and 44 parents of diabetic children (36 in England, 8 in Scotland)
- Adults with T1DM recruited were between the ages of 18 and 35
- The parents group only included parents of T1DM children under the age of 18
- The patient background data are presented in Tables 1 and 2
- Utility values from the final mixed model are presented in Table 3

Table 1: Patient Demographic characteristics for the General Public and Adult Patient Groups

	Adult Patients (n=51)	General Public (n=100)
Age Mean (sd)	27.6	43.0
Gender Male (%)	29 (57%)	39 (39%)
Ethnic Group		
Asian or Asian British	3 (6%)	3 (3%)
Black or British Black	2 (4%)	0 (0%)
White	45 (88%)	92 (92%)
Other	1 (2%)	5 (5%)

Table 2: Patient Demographic characteristics for Parents of Children/Adolescents with Diabetes

	Parents of Adolescent Patients (n=24)	Parent of Child Patients (n=20)
Children		
Child's Age Mean (sd)	15.1 (1.6)	9.9 (1.2)
Child's Gender (% male)	12 (50%)	13 (65%)
Child's Years of Education (sd)	10.3 (1.6)	4.9 (1.3)
Child's Ethnic Group		
Asian or Asian British	2 (8%)	1 (5%)
Black or British Black	1 (4%)	0 (0%)
White	18 (75%)	18 (90%)
Other	3 (13%)	1 (5%)
Parents		
Parent's Age Mean (sd)	43.0 (5.9)	41.5 (7.0)
Parent's Gender (% male)	4 (17%)	9 (45%)
Parent's Ethnic Group		
Asian or Asian British	2 (8%)	1 (5%)
Black or British Black	1 (4%)	0 (0%)
White	20 (83%)	17 (85%)
Other	1 (4%)	2 (10%)

Table 3: Standard Gamble Utility Results

Group's SG utilities	N	T1DM	T1DM Insulin free	T1DM reduced Insulin need	On-fusion	On-fusion with APR
Combined group	195	0.925	0.982	0.946	0.832	0.794
General public adults	100	0.903	0.976	0.929	0.789	0.744
Diabetic adults	51	0.938	0.985	0.955	0.859	0.825
Diabetic adolescents*	24	0.947	0.987	0.961	0.877	0.847
Diabetic children*	20	0.955	0.980	0.968	0.896	0.870

APR= Acute phase reaction; * =Interviews with parents

Limitations

- This survey was conducted in the UK and may not be generalizable to other populations
- The collection of the child and adolescent data depended upon parents' responses
- Another limitation perhaps reflects the nature of the valuation exercise itself. It is unknown as to whether or not the members of the general public recognized the health states as being depictions of T1DM. If the states were recognized by an individual, it is unknown how this fact would influence their rating behavior

Conclusion

- The aim of the study was to derive health state utilities for health states which describe infusion therapy for the treatment of T1DM. Quantifying the potential impact of undergoing infusion therapy (both in terms of possible treatment outcomes and associated burden) is a valuable exercise in its own right and these data will also be suitable for supporting the modeling of cost-effectiveness
- The SG utilities indicated the significance of positive treatment outcomes. A modest increase in utility was shown for the 'reduced insulin need' state by all groups. The prospect of becoming 'insulin-free' had a greater significance attached to it across all groups when compared to the base disease state
- Participants recognized the impact on quality of life of attending for infusion therapy
- The utilities from the different groups were very consistent. There was only one area of difference between the groups. This related to the values for infusion therapy whereby adults with diabetes rated the impact of infusion therapy as much lower than other groups. This may be because adults with diabetes are accustomed to receiving injections
- The societal values for diabetes health state descriptions reveal a significantly more negative assessment of disease burden when compared to those with direct experience of the condition. Patients and carers have learnt to cope and adjust to the demands of the disease which may explain why they report higher utilities