

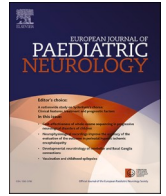
# ULRR

## Adolescents with Rett syndrome at critical care pathway junctures: Examining clinicians' decision to initiate invasive long-term ventilation

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Original article

## Adolescents with Rett syndrome at critical care pathway junctures: Examining clinicians' decision to initiate invasive long-term ventilation

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## ABSTRACT

**Background:** The initiation of invasive long-term ventilation (I-LTV) for an adolescent with Rett Syndrome (RTT) involves many serious bioethical considerations. In moving towards a more inclusive model of patient participation, transparency surrounding the main influencing factors around this decision is important.

**Objective:** We aimed to identify the main drivers influencing a clinician's decision to support initiation of I-LTV for an adolescent with RTT.

**Method:** We used an anonymous online vignette-based factorial survey. The survey was distributed internationally through eight professional multi-disciplinary organisations to reach clinicians working in paediatrics.

**Results:** We analysed 504 RTT vignettes completed by 246 clinicians using mixed effect regression modelling. The main three significant influencing factors identified were: parental agreement with the decision to support initiation, the family's support network, and proximity to a tertiary care centre. Additional comments from participants focused on family support, and the importance of on-going communication with the family.

**Conclusion:** As the rights of those with disabilities improve and participation of adolescents in decision-making becomes more established, effective communications with the family around goals of care and particular sensitivity and reflective practice around methods of consensus building will likely contribute to a positive decision-making process at this difficult time.

### 1. Background

Rett syndrome (RTT) is a complex genetic neurodevelopmental condition that occurs in approximately one in every 10,000–15,000 female births with very rare occurrence observed in males [1]. Symptoms vary between individuals and usually emerge prior to the child's second birthday [2]. These symptoms are most often characterised by motor and behavioural deterioration that precede the onset of abnormalities such as ataxia, repetitive and uncontrolled hand movements, acquired microcephaly, cognitive/language/speech impairments, as well as the development of other complex, comorbidities including seizures, scoliosis, gastrointestinal, and cardiac issues [3]. Many adolescents with RTT also experience serious respiratory issues, a leading cause of illness and mortality amongst this group [4]. Non-invasive long-term ventilation

(NI-LTV) is an established symptom management option for those experiencing chronic critical respiratory distress [5]. However, for some individuals with ongoing respiratory deterioration, progression to more permanent respiratory supports are required, and the transition from NI-LTV to invasive long-term ventilation (I-LTV) via tracheostomy requires further thoughtful consideration with particular regard to the consequences of the decision for adolescents living with RTT and their families. The life expectancy for many individuals with RTT is increasingly positive, however most experience multiple challenging associated comorbidities and require life-long care supports [3,4]. For the medical team, many bioethical factors require consideration. This includes clinical characteristics, family capacity, clinicians' experiences of similar cases, and available medical technology. The burden of care experienced by carers of a family member with a severe neurological

**Abbreviations:** AIC, Akaike's Information Criterion; ICC, Interclass Correlation Coefficient; I-LTV, Invasive Long-Term Ventilation; NI-LTV, Non-Invasive Long-Term Ventilation; QoL, Quality of Life; RTT, Rett Syndrome.

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impairment such as RTT is well documented [6]. This burden of care can impact on the psychological, physical, financial and logistical aspects of family day-to-day life [7,8]. As part of this role, primary carers are also often required to make serious medical decisions for their loved one [9]. For families faced with such serious decision-making responsibilities, being listened to, included, and supported by the medical team is a hugely important facilitator in their efforts to navigate through this challenging journey of care [10]. However, previous studies highlight how families instead often feel isolated and unsupported during these difficult decision-making periods [10]. Respectful open communication alongside consensus on goals of care between the patient, their family and clinicians are considered core facets underpinning compassionate modern medical care and treatment [11–13]. Identifying the main contributing factors to a clinician’s decision to support initiation in adolescents with a diagnosis of RTT provides a transparent foundation upon which to establish these positive relationships, this is the purpose of the current study.

This research was conducted as part of a larger programme of research, TechChild, funded by the European Research Council. This research aims to investigate at a broader level this important decision-making period and in doing so enhance understanding of the influences on a clinician’s decision to initiate life sustaining technology dependence for children with different complex illnesses.

## 2. Methods

### 2.1. Design

We used a vignette-based factorial survey to examine the main predictors of a clinician’s likelihood to support the initiation of I-LTV via tracheostomy for an adolescent with RTT. This survey was developed using clinical as well as factorial survey methodology literature in addition to earlier findings from the TechChild project [11,14]. An in-depth description of the development, pre-testing and piloting of the factorial survey tool is reported separately [15].

Ethical approval was received from the Trinity College Dublin Faculty of Health Science Research Ethics Committee (approval number: 190102) and the institutional data protection officer approved the study data protection impact assessment and deemed the study low risk.

For each participant, the final factorial survey comprised of eight

concurrent vignettes, comprising double-blinded interchangeable factors, which are described in Fig. 1.

Participants were asked to read and rate each vignette on a forced pseudo-Likert four-point scale of likelihood to support the decision to initiation I-LTV. Participants reviewed up to two RTT vignettes each [15]. Participants were given the option to provide open-ended feedback after their vignette response. Feedback during pretesting indicated this was an important option so that where participants found a decision difficult to answer they could contextualise their response or comment on other influencing factors not included in the vignette which they considered important. This qualitative data was examined using a simple descriptive approach with reporting based on the Critical Appraisal Skills Programme (CASP) checklist [16,17]. The RTT vignette can be seen in Fig. 2.

The vignette universe (i.e., the total number of combinations) for the RTT vignettes was 96 (2x2x2x2x3x2) [15]. Participants were also asked to complete a brief demographic section. The Consensus-Based Checklist for Reporting of Survey Studies as well as the Checklist for Reporting Results of Internet E-Surveys were used as a guide to report on the survey (CROSS) [18,19].

### 2.2. Participants and sampling

Non-probability purposive sampling was used. To be included in our study participants were required to be clinical professionals with experience caring for a child at the time when the initiation of life sustaining technology dependence is being considered. Participants were recruited virtually via eight international professional organisations from the areas of paediatric respiratory, palliative, and critical care. Organisations distributed the anonymous survey to all their members via email, website, or social media.

The survey was blinded at source and neither the research team nor the organisations were able to identify the participants. All potential participants were invited to click on a link that gave them access to the survey. The survey was constructed using Qualtrics survey software (Qualtrics, Provo, UT) [20]. The first page viewed by potential participants explained the purpose of the study and included a participant information leaflet and consent form. After reading the study information and clicking agree to the consent form, participants then progressed to the survey page. Distribution of the survey commenced in October

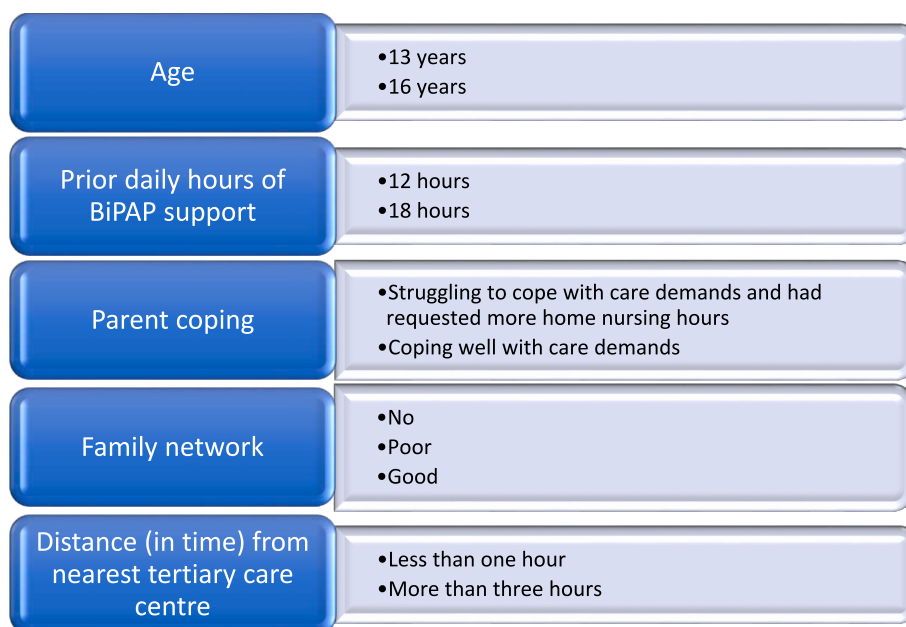


Fig. 1. Final RTT vignette content (Factors and levels for adolescent survey [15]).

A [Age] year-old with a history of Rett Syndrome is currently ventilated in the PICU and is difficult to wean from ventilation. This is the second time they have been ventilated in the PICU in the last year. This adolescent has been on BiPAP [Prior daily hours of BiPAP support] hours a day at home; there is an overall deterioration in their chronic respiratory condition. The degree of daily caregiving support for this adolescent's respiratory health increased in the month prior to admission, including increased nebulisation and suctioning. The family are receiving home care nursing hours, and, prior to the adolescent's current deterioration, the parents stated that they had been [Parent coping]

Consideration is now being given to continuous invasive long-term ventilation (LTV) via tracheostomy. The parents [Parent view] with the medical team on the need for initiating this treatment. There is a [Family network] family network of support around them. This adolescent and their parents live [Distance in time] from their nearest specialist care centre.

**Thinking about the above scenario, on a scale of 1-4, with 1 being extremely unlikely and 4 being extremely likely, how likely are you to support the initiation of continuous invasive long-term ventilation via tracheostomy for this adolescent?**

1. Extremely unlikely	2. Unlikely	3. Likely	4. Extremely likely
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*Please feel free to add comments regarding your choice of response*

Fig. 2. Vignette with interchangeable factors for RTT [15].

2021 and the link remained active until mid-January 2022. The recommendations of the RESTORE study were followed alongside the Qualtrics survey protection feature to validate entries and prevent bot completion and validate entries [21]. In addition, this study was carried out in accordance with The Code of Ethics of the World Medical Association for experiments involving humans (Declaration of Helsinki).

2.3. Data analysis

Descriptive analysis was completed to provide an overview of the participants' profiles. The factorial survey questions were compulsory and so there was no missing vignette data. In the optional demographic section, at the end of the survey, missing data ranged from 0 to 12.5% (age of participants). A review of this missing data did not identify any concerns or patterns. Fig. 3 shows the demographic questions that were asked of the respondents.

We performed mixed-effects modelling to identify the main

predictors (independent variables) on a clinician's likelihood to support the initiation of I-LTV for an adolescent (dependant variable) with RTT. All independent variables were carefully selected based on evidenced-based research, or previous research in the area and so all were included in the model. Thus, while each scenario will have included randomly interchangeable factors, clustering at the respondent level was a possibility and this was assessed using an assessment of the interclass correlation coefficient (ICC). Akaike's information criterion (AIC) was considered the most appropriate assessment of sensitivity analysis to compare predictor models due to the possible nested structure involved. The Likert scale responses were coded into a binary format (supportive of initiation vs not supportive of initiation) permitting a mixed effects binary logistic regression modelling approach. Categorical predictor variables were dummy coded and statistical analysis was completed using IBM SPSS Statistics for Windows, version 28 (IBM Corp., Armonk, N.Y., USA) [22]. We considered a two-tailed p-value less than 0.05 as indicative of statistical significance.

- Age
- Gender
- Do you consider yourself as belonging to any particular religion or denomination?
- Years' experience working with children with complex care needs
- Discipline/Job title
- Years in current position
- Country of employment
  - (Reported grouped by continent to preserve anonymity)
- Through which professional organisation did you hear about this survey?
  - (Validity check question)
- Any additional comments you would like to share
  - End of survey comment opportunity

Fig. 3. Demographic aspects of the survey [15].

In terms of the qualitative data, participants added open ended comments after 100 completed RTT vignette cases (out of a total of 504 RTT scenarios analysed). Most comments were brief with the majority comprising a single sentence or less. This qualitative response data was analysed using a simple inductive thematic analysis strategy informed by the Braun and Clarke (2006) framework [23]. The main purpose of providing an open-ended comments section after each vignette was to provide an opportunity for participants to comment on factors not included that they felt were important in forming their decision, or other information they considered important to share (such as their views on survey itself, and additional issues related to the topic).

### 3. Results

#### 3.1. Vignette analysis

We analysed 504 vignettes, completed by n = 249 clinicians (Table 1). Of these, three-quarters were medical doctors (n = 180) and nearly 14% (n = 32) nurses. Of those who provided demographic information, 59% (n = 138) were female with an overall mean age of 45.8 years (SD = 10.17). A little over 60% (n = 142) were employed in settings based in the United States of America and over half (52.8%; n = 124) indicated they maintained a religious affiliation.

The null (intercept only) model (Table 2) indicated significant variance between respondents, and the interclass correlation coefficient indicated that 27.9% of varies in responses lies between clinicians suggesting significant clustering at the participant level. The predictor model assessed the impact of a number of factors on the likelihood that clinicians would support the initiation of I-LTV for a child with RTT. The best fit model contained 10 independent variables. Three of the independent variables made a unique statistically significant contribution to the model (parents view; family network; distance). The strongest predictor of a clinician's support for initiation was the parent's view, recording an odds ratio of 12.9. This indicated that when a vignette

**Table 1**  
Profile of clinicians who completed RTT vignettes.

Profile variable	
Age (n = 223) <sup>a</sup>	Mean 45.84 years (SD = 10.17) (Median 44 years; 25–80 years <sup>b</sup> )
Years' experience with children with complex care needs (CCCN) (n = 230) <sup>a</sup>	Mean = 15.99 years; SD = 91 (Median = 15 years; 1–50 years) N (%)
Gender (n = 235)	
Female	138 (58.7%)
Male	93 (39.6%)
Non-binary	1 (0.4%)
Prefer not to specify	3 (1.3%)
Belonging to a religious denomination (n = 235)	
Yes	124 (52.8%)
No	96 (40.9%)
Not sure	9 (3.8%)
Prefer not to specify	6 (2.6%)
Profession (n = 232)	
Medical Doctor	180 (77.6%)
Registered Nurse/Nurse practitioner	32 (13.8%)
Respiratory Therapist	10 (4.3%)
Other members of the MDT <sup>a</sup>	10 (4.3%)
Current country of employment (n = 230) <sup>b</sup>	[Based on United Nations (UN) categorisation system]
North America	142 (61.7%)
Europe	59 (25.7%)
Oceania	15 (6.5%)
South America	7 (3.0%)
Asia	4 (1.7%)
Africa	3 (1.3%)

<sup>a</sup> Multi-Disciplinary Team: Includes Dietician, Pharmacist, Physician Associate, Physiotherapist/Physical Therapists, Senior Care Assistant.

<sup>b</sup> Responses collapsed from countries to continents (as per UN categorisation) to protect participants anonymity.

**Table 2**

Adolescent RTT vignette survey comparing the null/intercept model and model including predictors (Guided by LEVEL reporting [24]).

Vignette factors (level 1)	Value of category	Model 1: Null model (n <sub>1</sub> = 504; vignettes N <sub>2</sub> = 249 respondents)	Model 2 n <sub>1</sub> = 404 vignettes; n <sub>2</sub> = 198 respondents <sup>a</sup> )	P
		OR (95% CL)	OR (95%CL)	
		N/A		
Adolescent's age	16 years	N/A	0.767 (0.432-1.36)	0.362
	13 years			
BiPAP support	12 h	N/A	0.824 (0.459-1.477)	0.514
	18 h			
Parent coping	Coping well with care demands	N/A	1.248 (0.704-2.212)	0.447
	Struggling to cope with care demands and had requested more home nursing hours			
Parent view (on decision to initiate)	Agree	N/A	12.9 (CI 7.11-23.42)	<0.001
	Disagree			
Family network	A good (network)	N/A	2.55 (CI 1.4-4.65)	0.002
	No/A poor (network)			
Distance (from nearest tertiary care centre)	Less than 1 h	N/A	1.97 (1.11-3.52)	0.022
	More than 3 h			
Respondent factors				
Age (of clinician)		N/A	0.976 (0.911-1.046)	0.489
Gender	Male	N/A	1.336 (0.683-2.614)	0.369
	Female			
Religiosity	Yes	N/A	1.497 (0.774-2.896)	0.23
	No			
No of years working with children with complex medical needs		N/A	0.998 (0.928-1.074)	0.968
Intraclass Correlation Coefficient (ICC)		0.279 (<0.001)	NA	
Akaike Information Criterion (AIC)		2210.34	1920.49	

An intermediate model was build using only the vignette predictor variables however the model included was a better fit.

<sup>a</sup> Note that the number of respondents in this model is lower due to non-response to demographic variables.

noted a parent's support for initiation, respondents were nearly 13 times more likely to support initiation than for vignettes where parents did not support initiation I-LTV for their child. The odds ratio of 2.55 for family network indicated that when a good family network was reported in a vignette, respondents were 2.55 times more likely to support initiation than when no/a poor family network was reported. In addition, the model indicated that when a scenario described the family living within an hour of a tertiary centre, a clinician was 1.97 times more likely to indicate support for initiation than when the family lived more than 3 h

away.

### 3.2. Qualitative comments summary

Overall, the comments reflect the clinicians' focus on the family; an area also identified in the quantitative analysis. The sequelae of living with RTT was mentioned by many of the participants with reference to RTT prognosis as well as quality of life (QoL) for both the child and their family. QoL was the main topic that participants indicated they would have preferred more information on in the survey before making a decision. For some respondents, supporting the initiation of I-LTV meant it was considered more likely the adolescent would have better symptom management and thus QoL improvement for the adolescent as well as the wider family. For other respondents, there were concerns that initiation of I-LTV may lead to poorer QoL for the adolescent, because of the likelihood of the natural deterioration in their condition. Some respondents made reference to the provision of home care supports. Whilst not explicitly mentioned as an influencer, where a scenario indicated parents wanted initiation to proceed, home nursing support was noted as something the clinician would investigate in order to facilitate the parent's wishes.

Many respondents noted the importance of ongoing discussions with the family especially around developing a shared understanding of goals of care. A number of respondents recommended the involvement of palliative care services during the decision-making process, highlighting the importance of this specialist service during this difficult time. A few clinicians also explicitly mentioned the patient's own involvement in the decision-making process (if involvement was possible).

## 4. Discussion

This study examined the main influencing factors on a clinician's decision to support the initiation of I-LTV for an adolescent (13; 16 years) with RTT. This research is novel in that a factorial survey approach has not previously been used with this population of clinicians who work with adolescents with RTT requiring I-LTV to sustain life.

Increasingly, different jurisdictions have implemented laws that protect the rights of individuals with disabilities to make decisions, where possible, regarding their own health care (e.g. European Convention on Human Rights; the United Nations Convention on the Rights of Persons with Disabilities) [25,26]. Building on these legal obligations, much contemporary disability research aims to support and maximise a person's capacity, where possible, to make such decisions and, following, many healthcare settings policies have shifted towards establishing more inclusive models of decision-making [27,28]. In the paediatric setting, rapid technological advancements allow more children with serious conditions to survive PICU stays and live longer with more complex co-morbidities [29]. With these longer life expectancies and more complex conditions QoL is a particularly important factor to consider for these children and their families, especially for conditions like RTT, given the significant impact on both the child's development and the entire family [30,31]. As these children get older, decision-making regarding their care becomes more complex and approaches to participation and autonomy vary [32,33]. It is clear from existing literature that paediatric healthcare settings have overwhelmingly shifted towards family-centred shared decision-making policies and procedures; although the extent to which these are implemented on the ground is still debated [9,34–36]. Considering the cognitive, motor as well as language and speech impairments often experienced by children and adolescents with RTT, it is perhaps not surprising that child participation research focuses more on the enhancement of basic communication strategies [37,38]. With this in mind, the enhancement of appropriate decision-making participation opportunities for both the child/adolescent with RTT and their parents, requires compassionate, empathic healthcare environments based on respect and trust, especially when the decision is as consequential as the

initiation of life-sustaining technology dependence. However, as a core member of the shared decision-making model, there is still only scant research in paediatrics that investigates the influences on clinicians when considering this important decision; and this impedes transparency concerning how such decisions are made [14]. Similar factorial survey research with clinicians and adult clinical populations does highlight how nuanced such decision-making may be depending on the population and medical context [39]. For example, the primary concerns of clinicians in Drewniak et al's (2022) study examining the initiation of life sustaining technology for adults with cardiopulmonary failure focused on patients' age, treatment costs and care goals, comorbidities, as well as neurological outcome [39]. In contrast, a similar study examining commencement of life-sustaining technology dependence for young children with SMA type1 highlight that the parents' view was the primary deciding factor on a clinician's decision to initiate [40]. The identification of the main drivers behind a clinician's decision to initiate technology dependence for paediatric populations with RTT and other conditions is of much value in the clinical setting, as the findings can contribute to the development of more transparent and respectful healthcare environments that patients and their families can trust and participate in in a meaningful way. Rak and colleagues report that, in hospital settings that perform well in terms of LTV care and have optimal patient outcomes, where possible the child's view was considered in any decision-making and parents were actively involved in the care of their child, and considered a key part of the care team [41]. In line with this paediatric literature, the findings of this factorial survey also indicate that, clinical considerations aside, in cases of RTT, clinicians' decision-making does indeed centre around the family. The three main predictors: the parent's view on initiation, family network, and distance from a tertiary care centre, all point to clinician awareness and acknowledgement of the perceived challenges likely to be experienced by the family post-initiation, or as a result of their decision not to initiate. The emphasis in the qualitative findings concerning communication on goals of care as well as more information quality of life, alongside their comments regarding the availability of supports, further reflect this perspective and are also reflected in the literature in this area [30,31].

This international study included professionals from a range of disciplines and from different hospital settings, which contributes to a broader understanding of the influences underpinning a clinician's support of initiation. Demographic factors measured were not identified as contributing significantly to the model, however our qualitative findings indicated that the geographical setting of the participant plays an important role in terms of the availability of home care resources and palliative care services. These findings, alongside the significant contributing influence of the patient's proximity to a tertiary care centre, highlight the importance of resource availability and accessibility to the quality of a child's care, an issue evidenced clearly in the literature. Findings from paediatric and adult studies consistently point to the inequitable impact of location on healthcare provision [42,43]. The majority of participants in this study were employed in developed and well-resourced countries and the comments reflect this cohort. However, as many studies have highlighted, there is often a lack and disparity of specific skills within paediatric care teams between hospitals, across even the most resourced countries and in particular in rural areas, and this impedes the quality of service provision and care planning for children with complex needs and their families [36,44–46]. While the availability of community services may not directly influence a decision to initiate, the specialism and skills of outreach teams as well as palliative care services seem valued by the clinicians in this study in terms of establishing effective relationships with families and improving understanding and obtaining consensus around goals of care at times where difficult decisions are imminent. The comments from the minority of participants based in more under-resourced countries also point to many challenges that exist and this topic warrants further exploration. One of the leading causes of sudden mortality in individuals

with RTT is respiratory failure and, in practical terms, children highly dependent on I-LTV are likely to be at higher risk or requiring more resources (such as an additional back-up device) [4,47]. With this in mind, our finding that distance from a centre may be an influence on clinicians when considering initiation of technology dependence for individuals with RTT with a history of respiratory issues is understandable.

The survey was disseminated across a broad range of professional organisations, giving a broad perspective of this issue. However, the survey was anonymised at source and the majority of organisations only retained very limited information on their members (including membership number breakdowns). As a result, it was not possible to calculate response rate or the proportional participation of the target population. The high completion rate of those who accessed the survey suggests that the survey was appropriate in content and length and the small number of comments regarding the survey/methodology itself also reflect this point. The team made a concerted effort to gain a wide international reach through such organisations however the dominance of participants from the United States and Europe are also acknowledged. A more focused targeted survey examining countries with less developed healthcare structures would provide valuable context driven findings on what influences clinicians' likelihood to support I-LTV across more diverse geo-political settings.

Paediatric autonomy and the voice of the adolescent with disabilities in medical decision-making has become more of a focus in paediatric decision-making literature [32–34]. However, in this survey, there was clear consensus from clinical experts that including a vignette factor describing the adolescent's own view on initiation lacked external validity considering the likelihood of cognitive and communication difficulties for adolescents with RTT. As Hunt and colleagues observed in their study of paediatric pain in children with severe neurological impairment, knowing the child, experience of similar cases and knowledge of the science is often the most appropriate assessment and management, and this was the approach taken in the context of this study [48]. A small minority of respondents mentioned the adolescent's own view on initiation in their feedback acknowledging the importance of patient participation. However, this low number of comments concerning the child's view on the decision to initiate, also adds support to the research team's decision not to include the child's own opinion as an individual factor for RTT scenarios specifically given the design of the survey. However, the research team fully acknowledge that the perspective of the adolescent is extremely important. Communication strategies used with adolescents living with RTT continue to improve and there is a push for more inclusive practices where possible [38,49]. With this in mind, the voice and rights and involvement of the adolescent with a disability in medical decision-making will be considered in depth using a consultative approach in a future phase of the TechChild programme of research. Taylor (2006) notes, referencing research work in social work and care, that the factorial survey is a very useful tool to examining professionals' judgements on sensitive issues and this point has also been evidenced in this study. Further research, using this approach to explore clinicians' views on the inclusion of patients with cognitive impairments in decision-making, could also advance our understanding of this important topic [50].

## 5. Conclusion

RTT is a diagnosis that means life-long regular interaction with healthcare professionals and services for the individual and their carers. This study provides important insights into what influencing factors contribute to a clinician's likelihood to support or not support initiation of I-LTV for an adolescent with RTT. The factorial survey provides a useful approach to examining this sensitive topic in a transparent way that addresses many validity concerns of traditional survey methods. A clinician's decision to support the initiation of I-LTV for an individual with RTT who is in respiratory decline is one that ultimately considers

whether initiation is in the child's best interests and will lead to improvements or a decline in quality of life for the adolescent and their family. Where adequate resources and family supports are available the decision of whether to initiate or not is perceived by clinicians as more a family choice based on their goals of care as discussed with the medical team. These findings alongside findings from factorial surveys of different diagnoses will be collated with a breath of qualitative findings to improve our understanding of the influences on clinicians considering this important decision to initiate life-sustaining technology dependence.

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## Ethical approval

Ethical approval was received from the Trinity College Dublin Faculty of Health Science Research Ethics Committee (approval number: 190102).

## Declaration of competing interest

All authors declare no conflict of interest.

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