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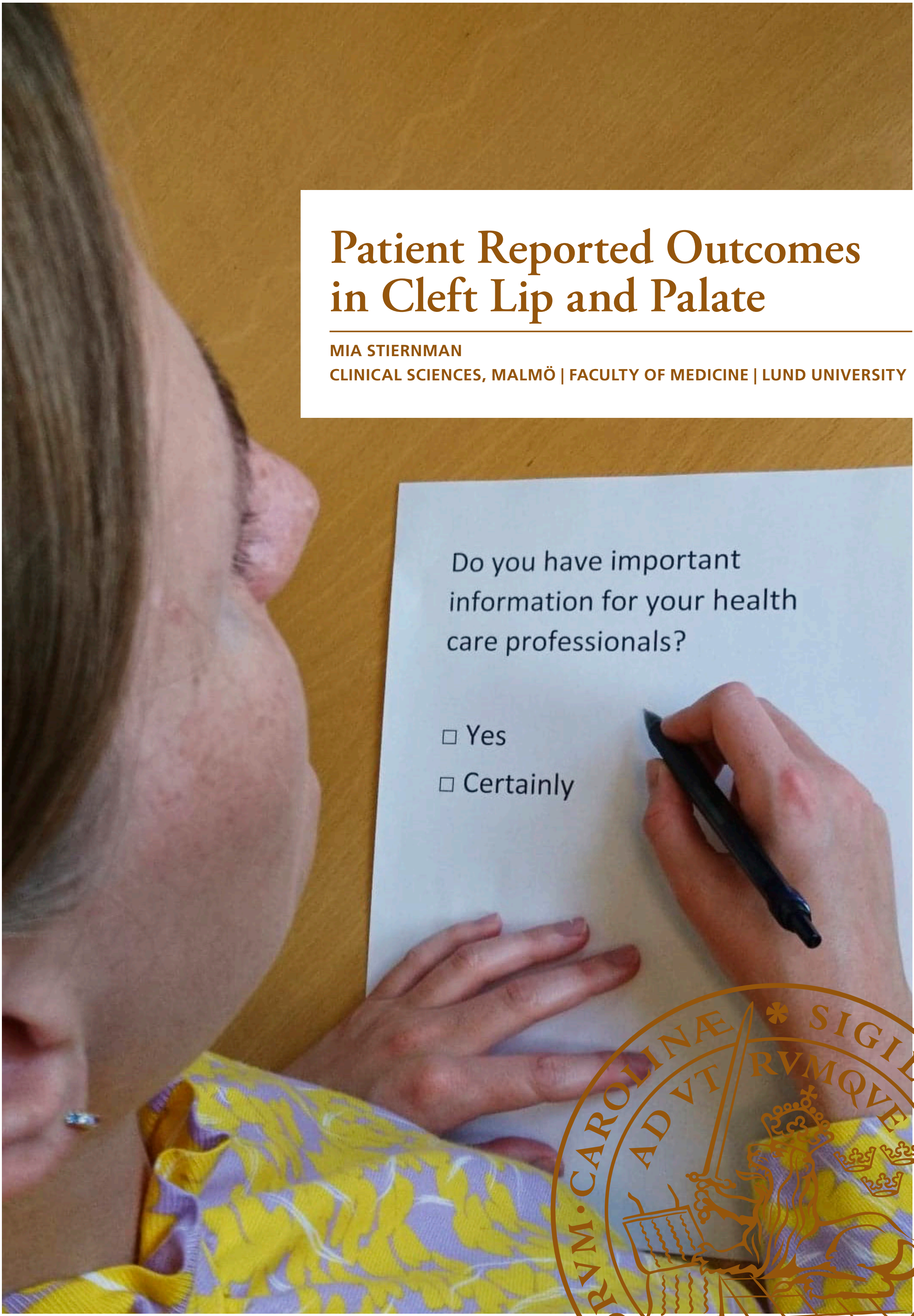
Patient Reported Outcomes in Cleft Lip and Palate

MIA STIERNMAN
CLINICAL SCIENCES, MALMÖ | FACULTY OF MEDICINE | LUND UNIVERSITY



MIA STIERNMAN is currently working as a surgical resident (ST-läkare) at the Department of Plastic and Reconstructive Surgery, Skåne Univeristy Hospital, Malmö, Sweden. In this thesis, patient reported outcomes in cleft lip and palate are studied. Questionnaire, focus group discussion and interview research methods were used. Patient reported outcomes are an important source of information to guide clinical work, for development of care and in research on cleft lip and palate.

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Patient Reported Outcomes in Cleft Lip and Palate

Mia Stiernman

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Abstract

Aim: Patient reported outcome measures (PROMs) quantify various domains of health related quality of life (HRQOL) from the patient perspective. The overall aim of this thesis was to investigate patient reported outcomes (PROs) in patients with cleft lip and/or palate (CL/P).

Method: PROs were investigated in the CL/P population with PROMs and interviews. Health care professional (HCP) experience of implementing PROs was investigated with focus group discussion. Patients who had been investigated for velopharyngeal dysfunction (VPD) completed a questionnaire on satisfaction with speech. Parents of children with CL/P and HCPs were interviewed regarding psychosocial and educational issues. The Cleft Hearing Appearance and Speech Questionnaire (CHASQ) was translated and implemented in 8 countries. CHASQ was tested in a control population and compared with results of the CL/P population. Results of CHASQ and CLEFT-Q were compared and patient opinion on the two PROMs were investigated.

Results: Most patients with VPD who underwent evaluation and treatment felt that surgery and speech therapy had improved their speech, but ultimately only approximately 50% of them were satisfied with the quality of their speech. Parental experience and views were diverse, ranging from the opinion that there were no specific problems related to the cleft - to a clear expression of both emotional and educational issues. The views and level of knowledge of HCPs also varied. All HCPs, however, wished for more information and training regarding psychosocial and educational issues and treatment. The CHASQ was translated into eight languages: Bulgarian, Estonian, Greek, Latvian, Macedonian, Romanian, Serbian and Swedish. Different levels of satisfaction between countries were revealed. There were patients in all countries who were less satisfied than expected and therefore should be identified for further investigation or treatment. Clinicians in multiple countries expressed the usefulness of CHASQ in their clinical work. It was perceived as useful, short, and easy to implement. CHASQ could be an effective instrument for collection of PROs on satisfaction with hearing, appearance and speech. Children and young people with CL/P were as satisfied with their appearance, hearing and speech as children and young people without CL/P. Scores from CHASQ and CLEFT-Q correlated well. Patients thought that CHASQ was easier to complete than CLEFT-Q. They liked CLEFT-Q more and thought that it better informed HCPs about their thoughts, opinions and feelings than CHASQ.

Conclusion: PROs in the CL/P population indicate a generally high level of satisfaction with outcome which is comparable to that of a control population in regard to satisfaction with hearing, appearance and speech. A PROM could be a useful instrument for improved communication between clinicians and patients and their families. Efforts for care and research in the CL/P population should include implementation of a CL/P specific and holistic PROM. Consensus on an international level of adaption of the same PROM and method for implementation is favourable.

Key words: Patient Reported Outcomes, Cleft Lip and Palate, Health Related Quality of Life

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Patient Reported Outcomes In Cleft Lip and Palate

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MADE IN SWEDEN

Science is a powerful force for change in modern society and plastic surgeons have a responsibility to shepherd that change with thoughtful advocacy and careful ethical scrutiny of their own behaviour (Sterodimas et al. 2011).

Table of Contents

Original studies

This thesis is based on the following original studies. They will be referred to in Roman numerals in this thesis.

Study I	Stiernman, M., K. Klintö, A. D. Al Qatani, B. Schönmeyr and M. Becker (2015). "Subjective outcomes after treatment for velopharyngeal dysfunction." J Plast Surg Hand Surg **49**(4): 198-203.
Study II	Stiernman, M., K. Österlind, N. Rumsey, M. Becker and M. Persson (2019). "Parental and Health Care Professional Views on Psychosocial and Educational Outcomes in Patients with Cleft Lip and/or Cleft Palate." European Journal of Plastic Surgery **42**(2): 325-336.
Study III	Stiernman, M., I. Maulina, I. Zepa, T. Jagomägi, N. Tanaskovic, P. Knežević, R. Velikova, Y. Anastassov, J. Radojićić, Z. Pesic, B. Trifunović, M. Drevensek, R. Spataru, T. Boljevic, R. Dimovska, S. Naumovski, N. Rumsey, F. Zucchelli, N. Sharratt, M. Argyrides, K. Klintö, M. Becker and M. Persson (2019). "Translation and Pilot Study of the Cleft Hearing Appearance and Speech Questionnaire (CHASQ)." European Journal of Plastic Surgery. https://doi.org/10.1007/s00238-019-01543-9
Study IV	Scores of the Cleft Hearing, Appearance and Speech Questionnaire (CHASQ) in Swedish patients with cleft lip and/or cleft palate and a control population. Stiernman, M., Klintö, K. Persson, M. Becker, M. In manuscript.
Study V	Comparison of scores from the CHASQ and the CLEFT-Q in Swedish patients with cleft lip and/or palate. Stiernman, M., Klintö, K. Persson, M. Becker, M. In manuscript.

Thesis at a glance

Study	Aims	Materials and methods	Main results
I	To evaluate satisfaction with speech, perceived speech quality, and perceived improvement in patients with velopharyngeal dysfunction (VPD).	114 patients with VPD completed a questionnaire on satisfaction with speech, perceived speech quality, and perceived improvement from treatment.	Seventy-one per cent of patients perceived their speech to be normal or slightly deviant, but only 55% were satisfied with their speech. Greater satisfaction with speech correlated with greater perceived improvement from surgery or speech therapy. Satisfaction with speech correlated significantly with perceived speech quality.
II	To investigate current beliefs and knowledge of health care professionals (HCPs) and parents of children with CL/P in southern Sweden regarding psychosocial and educational issues related to growing up with a visible cleft.	15 parents of children with a visible cleft and 10 HCPs were interviewed. Data was analyzed with thematic analysis.	The results revealed that parent experience and views were diverse - from no specific problems related to the cleft, to both emotional and educational issues. The beliefs and level of knowledge in HCPs also varied. All HCPs, however, wished for more information and training regarding psychosocial issues.
III	To translate Cleft Hearing Appearance and Speech Questionnaire (CHASQ) into eight European languages, to investigate whether levels of PROs in patients with CL/P were comparable across countries and to investigate clinician experience of the instrument.	Translation according to Mapi Research Trust guidelindes. CHASQ was implemented on 220 patients at routine visits to clinics. 16 HCPs participated in a focus group discussion regarding the strengths and challenges on the use of CHASQ.	CHASQ was translated into 8 languages: Bulgarian, Estonian, Greek, Latvian, Macedonian, Romanian, Serbian and Swedish. This study also revealed different levels of satisfaction with hearing, appearance and speech between countries. CHASQ was perceived as useful, short, and easy to implement. CHASQ could be an effective instrument for collection of patient reported outcomes.
IV	To investigate scores of CHASQ in patients with CL/P and a control population. The second aim was to compare CL/P and control population scores in this study with a British norm CL/P population.	64 patients with CL/P and 56 participants in a control population completed CHASQ.	Children and young people with CL/P in this study were as satisfied with their appearance, hearing and speech as children and young people without CL/P. Swedish CHASQ scores of both CL/P and non-CL/P related features of the face were similar to the mean scores in the British norm population.
V	To investigate the relation of scores between two existing CL/P specific PROMs, CHASQ and CLEFT-Q. The second aim of the study was to investigate patient opinion on the two instruments.	33 patients with CL/P answered CHASQ, CLEFT-Q and an extra survey regarding their opinions on the two PROMs.	All items on satisfaction with appearance of the face, specific parts of the face and speech in CHASQ correlated significantly with corresponding scales in CLEFT-Q. A majority, 15 patients (58%), liked CLEFT-Q more than CHASQ. Eighteen patients (69%) thought CHASQ was easier to complete and 19 (76%) that CLEFT-Q would better inform HCPs about their thoughts, opinions and feelings.

Abbreviations

ADHD	Attention Deficit Hyperactivity Disorder
CAT	Computerised Adaptive Testing
CHASQ	Cleft Hearing Appearance and Speech Questionnaire
CL/P	Cleft lip and/or palate
COHIP	Child Oral Health Impact Profile
CTT	Classical Test Theory
HCP	Health Care Professionals
HRQOL	Health Related Quality of Life
ICS	Intelligibility in Context Scale
ICHOM	The International Consortium for Health Outcomes Measurement
ISOQOL	International Society for Quality of Life Research
NOSE	Nasal Obstruction Symptom Evaluation Scale
PRO	Patient Reported Outcome
PROM	Patient Reported Outcome Measure
RMT	Rasch Measurement Theory
SLP	Speech and Language Pathologist
SWA	Satisfaction With Appearance Questionnaire
VPD	Velopharyngeal Dysfunction

Introduction

The introduction to this thesis first describes the patient population; the diagnosis cleft lip and/or palate (CL/P) and the treatment and follow-up program in Sweden. Health related quality of life (HRQOL) and patient reported outcome (PRO) is then defined and related to the patient population. An overview of earlier work on HRQOL and PROs in the CL/P population is presented. Finally, the patient reported outcome measures (PROMs) used in the studies comprising this thesis are presented in greater detail.

Cleft lip and/or palate (CL/P)

CL/P is the most common congenital craniofacial malformation affecting approximately two in 1000 live births in Sweden (Hagberg et al. 1998). There are many phenotypes and combinations of cleft of the lip, alveolus and palate. An updated classification is offered by Allori et al. (2017) and all abbreviations and descriptions of phenotypes in this thesis will follow the recommendations in that publication. Most common CL/P types and treatment in Sweden is presented in Table 1. CL/P may affect patient appearance, speech, hearing, occlusion, dentition and eating function to different degrees depending on the severity and differing anatomy involved. In approximately 20-30% of cases CL/P is associated with other birth defects, additional cognitive of psychological conditions or is part of a syndrome (Hagberg et al. 1998, Feragen et al. 2014). Treatment outcomes may be affected negatively by an additional condition or developmental delay (Feragen and Stock 2014, Feragen et al. 2014). The treatment of CL/P consists of reconstructive plastic surgery, and most often, treatment with a speech and language pathologist (SLP), an ear, nose and throat specialist, an orthodontist, psychologist or psychotherapist, and a specialized nurse. Most CL/P-centres have standardized treatment protocols for treatment indication and timing. However, techniques and timings of treatment vary in different parts of the world, as well as within Sweden.

Table 1. Common CL/P types and treatment in Sweden.

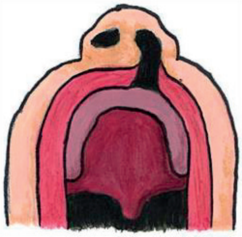
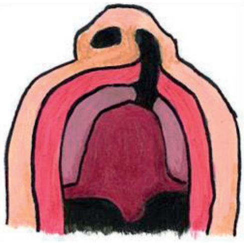
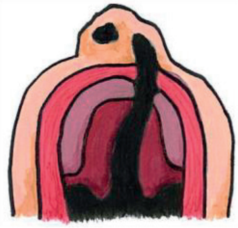
	Schematic anatomy	Time of diagnosis	First months after birth	Approximately 3 months of age
Cleft palate only (CP)		Information to family within first weeks after birth about diagnosis and treatment plan. Information on special feeding needs if indicated. Meeting with specialists involved in treatment plan.		
Cleft lip only (CL)			Tape and hook to lift lip and nose.	Lip repair surgery
Cleft lip with cleft alveolus (CL+A)			Tape and hook to lift lip and nose.	
Cleft lip and palate (CL+P)			Tape and hook to lift lip and nose. Palate plate if indicated.	
Bilateral cleft lip and palate (bCL+P)				

Table 1. Common CL/P types and treatment in Sweden, continued.

	4-24 months of age	2-6 years of age	Approximately 7-13 years of age	Approximately 13-19 years of age
Cleft palate only (CP)	Palate repair surgery in one stage at 9 to 12 months of age or two stage repair of soft palate at 6 months and hard palate at 24 months of age. Grommets inserted during surgery if indicated.	Speech and language treatment (if indicated). Velopharyngeal flap, secondary palate muscle repair or buccal flap to improve speech (if indicated).	Orthodontic treatment with expansion of upper jaw may be needed.	
Cleft lip only (CL)				Lip scar correction. (Secondary surgery if indicated)
Cleft lip with cleft alveolus (CL+A)			Braces or orthodontic treatment with expansion of upper jaw may be needed. Alveolus repair surgery with secondary bone graft in mixed dentition.	Correction of nose or lip. (Secondary surgery if indicated)
Cleft lip and palate (CL+P)	Palate repair surgery in one stage at 9 to 12 months of age or two stage repair of soft palate at 6 months and hard palate at 24 months of age. Grommets inserted during surgery if indicated.	Speech and language treatment (if indicated). Velopharyngeal flap, secondary palate muscle repair or buccal flap to improve speech (if indicated).		Correction of nose, lip or jaw. (Secondary surgery if indicated)
Bilateral cleft lip and palate (bCL+P)				

CL/P treatment in Sweden

The treatment of patients with CL/P varies slightly among centres in Sweden. The main difference is the cleft palate surgery, which is performed in one stage at two centres (Malmö and Linköping) and performed in two stages at four centres (Gothenburg, Stockholm, Uppsala and Umeå) (Becker and Klintö 2019). The essential treatment program is described in Table 1. All centres follow a standardised follow-up program. Children visit their regional centre regularly to monitor treatment results, growth of the parts of the face affected by CL/P, dentition/occlusion, speech and hearing. The visits occur approximately every three years up to 19 years of age and are attended by multiple health care professionals (HCPs) from different disciplines. At the follow-up visits children and their families are informed about treatment progress and have the opportunity to ask questions. If necessary, the HCPs can discuss and alter the treatment plan according to the child's status. The follow-up visits could also be an opportunity for collection of PROs (Arora and Haj 2016).

The Swedish CL/P registry

The Swedish CL/P registry was appointed as a national quality registry in 2016 by the National Board of Health and Welfare (In Swedish: Socialstyrelsen) and the Swedish Association of Local Authorities and Regions (In Swedish: Sveriges Kommuner och Landsting) (Becker and Klintö 2019). The aim of the registry is to compare results and learn from the different CL/P teams in Sweden; as listed below.

- To evaluate results of different treatment methods by assessing the development of appearance, dentition/occlusion and speech.
- To ensure equal treatment for children and adolescents born with CL/P in Sweden.
- To facilitate cooperation between CL/P centres in Sweden.
- To improve treatment methods of CL/P.

Results from CL/P centres regarding type, timing and number of treatment procedures and complications are registered. Treatment results are registered at 18 months and five, 10, 16, and 19 years of age and are reported annually. As yet, only one PROM is included in this registry; the Intelligibility in Context Scale (ICS) (McLeod et al. 2015), which is completed by parents of patients with CL/P at five and 10 years of age. The registry is a possible platform for further collection and future evaluation of PROs in Sweden.

The International Consortium for Health Outcomes Measurement (ICHOM) has set up a multidisciplinary and international working group to develop a standard set of outcome measures in CL/P care (Allori et al. 2016). The goal of the collaboration was to produce a minimal set of outcome measures for all CL/P teams to collect. With standard outcome measures and PROMs, teams can compare and benefit from each other to improve CL/P care as an international CL/P collective (Voineskos et al. 2018, Dobbs et al. 2019, Geoghegan et al. 2019). PROMs in the standard set included by the ICHOM working group are various CLEFT-Q scales and three additional PROMs – Child Oral Health Impact Profile (COHIP) (Broder et al. 2007), Nasal Obstruction Symptom Evaluation scale (NOSE) (Stewart et al. 2004) and ICS (McLeod et al. 2015).

Health Related Quality of Life (HRQOL)

HRQOL is defined by the International Society for Quality of Life Research as 'the functional effect of a medical condition and/or its consequent therapy upon a patient. HRQOL is thus subjective and multidimensional, encompassing physical and occupational function, psychological state, social interaction and somatic sensation' (ISOQOL 2019). Various aspects of HRQOL may be affected by CL/P and treatment thereof. High HRQOL is one of the most important goals in the treatment of CL/P, as clearly expressed by Klassen et al. (2012).

Patient Reported Outcome Measures (PROMs)

PROMs quantify various PROs and concepts of HRQOL from the patient perspective. Patient opinion and perspective are central in evaluating results of CL/P treatment since many interventions focus on increasing quality of life and improving patient self perception (Semb et al. 2005, Wormald and Rodrigues 2018, Dobbs et al. 2019, Geoghegan et al. 2019). Implementing PROs into standard clinical practice is in line with the paradigm shift and ethical approach of patient centred care, which is endorsed both in Sweden (Swedish Association of Local Authorities and Regions 2015, National Board of Health and Welfare 2016) and internationally (Black 2013, WHO 2019). It is also a way to improve and to compare quality of care between institutions and between different patient groups (Black 2013). PROMs also have the potential to facilitate shared decision making between patients and clinicians, guide improvement of cost-effectiveness in plastic and reconstructive surgery (Dobbs et al. 2019), improve patient-doctor communication and in addition, enhance patient satisfaction (Wormald and Rodrigues 2018). There was no internationally accepted CL/P specific and holistic

PROM to implement at CL/P centres until 2018 and the publication of the study protocol of the CLEFT-Q project (Wong Riff et al. 2017), and the psychometric findings of the international field test of CLEFT-Q (Klassen et al. 2018).

Psychometric methods

Psychometrics is the science of quantitatively measuring abstract and theoretical concepts in psychology, education and social sciences (Cano et al. 2009). In earlier publications the terms concept, domain and construct have been used interchangeably (Cano et al. 2009, Nelson 2009, Cano and Hobart 2011, Reeve et al. 2013, Dobbs et al. 2018, Stock et al. 2018). Instruments used for measuring psychological constructs are for example rating scales, questionnaires or PROMs. The majority of PROMs in use in plastic surgery today have been made and tested with Classical Test Theory (CTT) (Cano et al. 2009, Cano and Hobart 2011). The score produced by a CTT PROM is ordinal-level data, and intervals on different parts of the scale do not necessarily imply equal change in the measured construct. For example, the satisfaction with appearance of a patient who has undergone a surgical intervention can increase from ‘not satisfied’ to ‘moderately satisfied’. For another patient his or her satisfaction with appearance may increase from ‘moderately satisfied’ to ‘very satisfied’ after a surgical intervention. Both patients will have increased their satisfaction with one ordinal-level step on the measuring scale, but it will not be clear if their satisfaction with appearance increased equally. Rasch Measurement Theory (RMT) has been presented as an alternative way of measuring PRO in health care in general as well as in plastic and reconstructive surgery (Cano et al. 2009, Cano and Hobart 2011, Dobbs et al. 2018, Voineskos et al. 2018, Wormald and Rodrigues 2018). RMT PROMs produce interval-level data where differences in scores are equal across a scale. Further distinctions between CTT and RMT are listed in Table 2.

Table 2. Strengths and limitations of PROMs developed with Classical Test Theory and Rasch Measurement Theory (Cano et al. 2009, Cano and Hobart 2011, Dobbs et al. 2018, Voineskos et al. 2018, Wormald and Rodrigues 2018).

	Classical Test Theory	Rasch Measurement Theory
Strengths	Scores can easily be computed without conversion tables.	Produces interval-level score data from ordinal-level item data. Allows generation of a final score from subsets of items in a scale and computerised adaptive testing. Allows for missing data. Precise enough for clinical decision making on the individual level.
Limitations	Produces ordinal-level data.	Complex mathematical model. Requires specialised software or conversion table. Greater development costs due to large samples of study participants and high mathematical expertise in development team.

Quality of PROMs

High quality of PROMs ensures that research and clinical work on PROs is valid (Aaronson et al. 2002, Dobbs et al. 2018, Wormald and Rodrigues 2018). High validity implies that the test measures what it purports to measure and has low systematic error. High reliability is also essential in order to ensure low random error of the test. Responsiveness is the characteristic that enables the test to detect a change after an intervention (Aaronson et al. 2002). There are minimum standards recommended by ISOQOL to guide the choice and assessment of which PROMs to use in clinical practice and research (Reeve et al. 2013). The points included in the minimal standards are summarised in Table 3. PROMs, which do not have empirical evidence to support high quality on each point, may still be the best option for research in a specific study due to lack of better instruments. The validity of a PROM may be strengthened over time as the instrument is more extensively studied and tested (Reeve et al. 2013).

Table 3. Summary of points of evaluation of a PROM based on Reeve et al. 2013.

1. Conceptual and measurement model	Documentation about which constructs the instrument measures and which population it is intended for.
2. Reliability	The degree to which the PROM is free from random error. Reliability can be tested with internal consistency, test-retest reliability (Chronbach's alfa should be >0.70) or item response theory (person separation index should be >0.70). For a multi-item PROM, internal consistency is a measurement of how well the items measure the same construct. Test-retest reliability measures how stable the PROM measures a construct over time or between respondents when other conditions remain the same.
3. Validity	The degree to which the PROM is free from systematic error; measures what it is supposed to measure.
3a. Content validity	How well the PROM includes all relevant parts of the measured construct. This can be tested with qualitative interviews with patients in the intended target population.
3b. Construct validity	How well the PROM corresponds with other PROMs, observations and theoretical hypotheses of the same construct.
3c. Responsiveness	How well the PROM can detect significant change of the measured construct over time. This can be tested with a PROM before and after an intervention.
4. Interpretability of scores	The PROM should have documentation to aid interpretation of the scores produced. For example, norms of the target population or a control population, and cut offs for scores above or below the norm, indicating pathology.
5. Translation of the PROM	Documentation of translation process and testing of the PROM in the target language. This can be tested qualitatively, with cognitive testing for example; interview with patient for identification of unclear wording or linguistically or culturally ill suited text.
6. Patient and investigator burden	The burden of completing and administrating the PROM should be considered in relation to the intended study or clinical protocol. The level of literacy needed to complete the PROM should be documented.

Level of specificity of PROMs in CL/P HRQOL research

Both generic and disease specific questionnaires have been used to explore PROs in populations with CL/P. A generic questionnaire is one that is intended to be able to be used in various populations. One drawback is that they might not include relevant questions important to individuals with CL/P (Cano and Hobart 2011, Ricketts et al. 2016, Wormald and Rodrigues 2018). A disease specific questionnaire is made specifically with the target population in mind. On the other hand, a drawback of disease specific questionnaires is that they are hard to compare with norm values of individuals born without CL/P (Cano and Hobart 2011, Herkrath et al. 2015, Wormald and Rodrigues 2018). It is likely that studies of both disease specific and generic PROMs are necessary for a better understanding of the research field (Fitzpatrick et al. 1999, Cano and Hobart 2011, Crerand et al. 2017). The lack of comparisons of CL/P related outcomes with those in norm populations has been pointed out in earlier studies (Berger and Dalton 2011, Feragen et al. 2015, Stock and Feragen 2016, Stock et al. 2018). Data from different perspectives is also important in order to obtain an in depth picture of the patient situation (Thomas et al. 1997, Feragen et al. 2017). It is therefore necessary to combine objective of treatment outcomes with assessments of professionals, parents, as well as patient reported data in clinical routine (Fitzpatrick et al. 1999).

Impact of CL/P on HRQOL

Various aspects of patient HRQOL may be affected by the CL/P diagnosis and treatment protocol (Stock et al. 2019). Satisfaction with hearing, appearance and speech is interdependently connected with patient HRQOL but remains to be further investigated (Rumsey and Harcourt 2014, Stock and Feragen 2016). For decades, patient-reported data regarding satisfaction with appearance and speech has been shown to be a useful indicator for the need of psychological evaluation and intervention (Richman 1983, Thomas et al. 1997, Berger and Dalton 2011, Feragen et al. 2017). Reviews conclude that patients with CL/P are, in general, well adjusted and have no major psychosocial problems (Hunt et al. 2005, Stock and Feragen 2016). However, several concerns in specific domains of HRQOL became evident when examined in greater detail rather than as part of a wider HRQOL concept. These domains were: behavioural problems, satisfaction with facial appearance, depression and anxiety (Hunt et al. 2005), concerns about visual difference and experience of bullying (Sharif et al. 2013), psychological health and vitality (Herkrath et al. 2015), dissatisfaction with appearance, specific deficits in cognitive and language development, raised levels of self-consciousness, and anxiety (Stock and Feragen 2016).

Even though some domains of HRQOL seem to be affected on a group level in the CL/P population, it is clear that many individuals cope effectively with the above-mentioned challenges. Some also turn their unique experiences and viewpoints into positive insights and personal strengths (Strauss 2001, Feragen 2012). Increased research focus on positive outcomes and strengths displayed by people with CL/P has been called for (Strauss 2001, Stock et al. 2018). Studies focusing on positive outcomes have, thus far, produced insights on having a facial difference such as; participants perception of improved communication abilities, service to others, observational skills, inner strength, abilities to question society, a valued social circle, perception of being normal because of the difference (Eiserman 2001), increased value in diversity and individuality, as well as positive psychological growth (Stock et al. 2016). Specific domains in which people with CL/P have reported better HRQOL than a control population are; non-cleft related appearance, over all appearance, higher perception of social environment (Berger and Dalton 2009), more positive functioning in and perception of friendships, higher levels of social acceptance, more positive self-perceptions of appearance, and less emotional distress (Feragen et al. 2010).

Results from studies on HRQOL in the cleft population appear inconsistent. In many domains, results vary considerably both within and between studies, leading to conflicting conclusions. Qualitative studies shed light on the normally occurring complex psychological processes and large individual variation of HRQOL within the same domain (Stock et al. 2016, Kappen et al. 2019). It may be helpful to think of a CL/P as an underlying psychological stressor, which interacts with different psychological domains to a varying degree depending on personal disposition and age (Stock et al. 2016, Stock et al. 2018).

A few important psychological concepts which have been mentioned and studied in relation to individuals with CL/P are; adjustment, resilience and coping. Adjustment is a broad term describing the process of adapting and adjusting to a circumstance to be able to function adequately. Adjustment has been described as fluctuating and difficult to define or capture in a comprehensive way (Stock et al. 2018). For people with CL/P, good adjustment can be seen as one of the main goals in treatment - that the person can function adequately in his or her life despite possible appearance or speech differences (Feragen 2012, Stock et al. 2018). Resilience is the ability to maintain a high QOL or adjustment despite adversity or crisis (Luthar et al. 2000). Level of resilience has also been associated with satisfaction with appearance and speech in people with CL/P (Feragen et al. 2009). Coping relates to a specific stress and the conscious efforts that an individual makes to deal with that stress (Lazarus and Folkman 1984). Which coping style a person with CL/P uses has been seen to have an effect on the individual's as well as the family's level of psychosocial adjustment (Baker et al. 2009, Berger and Dalton 2011, Moss and Rosser 2012). It is also important to keep in mind that the above-mentioned concepts are closely related and that they interact with the occurrence of CL/P on an individual, social and sociocultural level (Feragen 2012, Stock et al. 2016, Stock et al. 2018).

When a patient has a high HRQOL, good function of speech, good occlusion and hearing and the patient is satisfied with appearance, no treatment is necessary. When a patient presents with issues related to treatment results or unacceptable function of speech, occlusion or hearing, and an appropriate treatment intervention is available, that treatment should be offered to the patient. When a treatment issue does not have an appropriate treatment intervention to increase HRQOL, this has to be clearly communicated to the patient and alternative pathways of referral presented, such as psychological counselling and treatment.

Relation between hearing, appearance, speech and HRQOL in individuals with CL/P

Hearing

Feragen et al. found that hearing was not an influential background variable in relation to speech, language, reading and psychological adjustment in a CL/P population (Feragen et al. 2017). This is in accordance with quantitative results from a study by Oosterkamp et al. (2007). However, there are indications that hearing impairment may affect HRQOL in patients with CL/P as in the qualitative section of this study, participants did in fact report concerns regarding hearing which were assumed to impact on HRQOL. Furthermore, hearing difficulties related to otitis media with effusion have been reported to be associated with social impairment in children with CL/P (Tierney et al. 2015). In addition, in one study, adults with CL/P reported that hearing difficulties affected their social integration greater than the cleft itself (Stock et al. 2016).

Appearance

Satisfaction with appearance in individuals with CL/P generally correlates with self-reported psychosocial functioning, wellbeing and HRQOL (Thomas et al. 1997, Oosterkamp et al. 2007, Feragen and Borge 2010, Feragen et al. 2010, Feragen et al. 2015, Feragen and Stock 2016). High satisfaction with appearance and speech, and low amount of reported teasing has also been associated with higher psychosocial resilience in patients with CLP (Feragen et al. 2009). As shown in the same study, visibility of the cleft did not correlate with the level of resilience. A further study concluded that patient social experience was the most influential factor for psychosocial adjustment, followed by level of perceived speech difficulties, level of satisfaction with appearance and coping style. Cleft type, however, was not a predictor of adjustment (Berger and Dalton 2011).

It has been stated that patient opinion on appearance is more important for patient wellbeing than the objective result, or professional or lay-man-rating (Richman 1983, Moss 2005, Feragen et al. 2010, Mani et al. 2010, Feragen et al. 2015, Pausch et al. 2016, Stock and Feragen 2016, Mulder et al. 2018). In a study design used by Richman (1983), self-reports from patients on social adjustment were compared to structured interview outcomes with a psychologist using objectively categorized response choices. The author suggests that the study results strengthen the validity of patient-reported data since objective levels of social adjustment correlated with the PRO (Richman 1983).

Research on the relation between satisfaction with appearance and HRQOL is not, however, unanimous. Feragen and Stock (2016), showed that the correlation between satisfaction with appearance and risk of low social, cognitive, emotional and behavioural adjustment was weak or insignificant for 10-year olds. Rather, the risk for low social, emotional and behavioural adjustment and dissatisfaction with appearance was correlated with the presence of additional conditions, such as developmental delay, learning difficulties, autism spectrum disorders or attention deficit hyperactivity disorder (ADHD) (Feragen and Stock 2016). At 16 years of age, however, an association was found between dissatisfaction with appearance and elevated risk for low emotional adjustment in both CL/P and non-CL/P participants (Feragen et al. 2015). The risk of low adjustment, however, seemed to be specific to a studied domain rather than to HRQOL in general. For example, low satisfaction with appearance affected the domain of emotional adjustment, but this effect did not spill over into other domains of adjustment. The authors also concluded that emotional and appearance related distress was common in both CL/P and non-CL/P participants at 16 years of age.

In summary, the relation between HRQOL and objective appearance or cleft type is unclear. Self reported satisfaction with appearance, however, has an influence on HRQOL. The effects of self reported satisfaction with appearance on HRQOL may vary with age and results may also vary depending on which specific domains of HRQOL are measured.

Speech

Earlier research has reported that satisfaction with speech correlates with psychological adjustment (Berger and Dalton 2011) rather than with results of perceptual speech assessment by speech language pathologists (Havstam et al. 2008, Feragen et al. 2017). No correlation was found between HRQOL and satisfaction with speech in quantitative results in a study by Oosterkamp et al. (2007). In the qualitative section of that study, however, participants did report concerns regarding speech, which were assumed to impact on HRQOL. Patient reported attitude toward communication has been correlated with results of perceptual assessment of speech in patients with CL/P (Havstam et al. 2011). In this study, patients who had a higher quality of speech also had a more positive attitude toward speaking.

In conclusion, professional opinion on quality of speech has been shown not to correlate particularly well with HRQOL. Patient satisfaction with speech has non-the less, shown to have a clearer association with HRQOL and psychological adjustment. Patient-reported data regarding satisfaction with speech, as with appearance, could therefore be a useful indicator for the need of psychological evaluation and intervention (Berger and Dalton 2011, Feragen et al. 2017).

Limitations of earlier research on patient reported outcomes (PROs) in patients with CL/P

One obstacle associated with PRO analysis in research regarding CL/P, is the lack of a widely accepted measurement instrument. In a review of current PRO HRQOL studies, it was found that most PROMs had been used in one study only (Klassen et al. 2012). Neither did the PROMs meet scientific standards of reliability, validity, and responsiveness (Eckstein et al. 2011). Another review of PROs in plastic surgery revealed that almost all disease-specific measures were ad-hoc measures produced for a particular study (Cano et al. 2004). Study results are therefore difficult to compare, draw conclusions from, and to build upon (Stock and Feragen 2016).

There are a number of biases related to PRO concerning patient satisfaction of which researchers need to be aware. For example, even though the clinical outcome of oral health, dentofacial form, and quality of bone grafting were disappointing, almost all patients and parents were satisfied or moderately satisfied with outcome of treatment (Williams et al. 2001). It was hypothesized that parents have great trust in HCPs but lack comparative knowledge of CL/P treatment and outcome. In the Eurocleft-study, few patients or parents were dissatisfied with overall results of treatment despite rather disappointing objective ratings of outcome (Semb et al. 2005). Although 65% reported satisfaction, further treatment was still desired. Despite 87% of patients in another study being satisfied with overall cleft-care, 37% of patients and 60% of parents nonetheless put forward suggestions for further treatment (Turner et al. 1997). The authors proposed that families who had received care from CL/P teams might report high satisfaction with treatment due to gratefulness and also to justify their emotional investment in the treatment. It has been suggested that over the years, older patients could have repressed or forgotten the painful feelings experienced when growing up with CL/P. In retrospective studies this could lead to higher reports of satisfaction. The fear of more surgical interventions could also result in patients reporting higher satisfaction with appearance (Broder et al. 1992). This hypothesis would however only apply to a subpopulation since a majority of patients, in the before mentioned studies, asked for and made suggestions for additional interventions. Further limitations of the use of PROMs in general is the added administrative burden and risk of exclusion of participants with low literacy (Wormald and Rodrigues 2018).

Specific PROMs used in this thesis

There are multiple PROMs in use in CL/P research. Those used in this thesis will be presented in greater detail.

Malmö speech questionnaire

Malmö speech questionnaire contained questions concerning the patient’s background, satisfaction with speech, perceived speech quality, and perceived improvement from treatment. It can be found in Appendix I. An evaluation of Malmö speech questionnaire based on points from Reeve et al. 2013 can be found in Table 4.

Table 4. Evaluation of Malmö speech questionnaire based on points from Reeve et al. 2013.

1. Conceptual and measurement model	Malmö speech questionnaire is an ad hoc questionnaire with no prior documentation of constructs measured or intended target population.
2. Reliability	There is no testing of questionnaire properties regarding test re-test reliability or internal consistency. The questionnaire prossibly measures several constructs.
3a. Content validity	Not evaluated.
3b. Construct validity	Not evaluated.
3c. Responsiveness	Not evaluated.
4. Interpretability of scores	No norms have been evaluated.
5. Translation of the PROM	Questionnaire was developed in Swedish. No other translations are avaliable.
6. Patient and investigator burden	Patients can complete the questionnaire in approximately five minutes. The level of literacy needed to complete the questionnaire has not been evaluated.

CHASQ

The psychometric properties of CHASQ have been evaluated with CTT. CHASQ and its predecessor, Satisfaction With Appearance Questionnare (SWA) have previously been used in clinical research in Europe (Berger and Dalton 2009, Feragen et al. 2009, Feragen and Borge 2010, Mani et al. 2010, Mani et al. 2013, Feragen et al. 2015, Feragen and Stock 2016, Crerand et al. 2017). The score for each item ranges from zero to 10 points. CHASQ in Swedish and English and the User Guide for the CHASQ can be found in Appendix II-IV. An evaluation of CHASQ based on the points from Reeve et al. 2013 can be found in Table 5.

Table 5. Evaluation of CHASQ based on points from Reeve et al. 2013.

1. Conceptual and measurement model	Documentation is available, however not published, regarding the development and usage of CHASQ (Cleft Psychology Clinical Excellence Network 2015). CHASQ is a modified version of the SWA. SWA was designed by the Cleft Psychology Special Interest Group, Craniofacial Society of Great Britain and Ireland specifically for patients with facial disfigurement (Emerson et al. 2004).
2. Reliability	There are two factor loadings within CHASQ (Cleft Psychology Clinical Excellence Network 2015). Factor 1 loading items consist of nine items regarding features typically affected by a cleft and factor 2 loading items consist of six items regarding features not typically affected by a cleft. Therefore, CHASQ produces two scores, sum of factor 1 loading items (total features 1) and sum of factor 2 loading items (total features 2). The internal consistency within the two factor loadings have been tested with Cronbach's alfa; factor 1 loading features 1 (0.90) and factor 2 loading items (0.83). Test-retest reliability has not been evaluated for CHASQ.
3a. Content validity	Content validity has not been evaluated with qualitative interviews with members of the patient population.
3b. Construct validity	CHASQ has shown construct validity since the score of factor 1 loading items after orthognatic surgery improved while score of factor 2 loading items did not significantly improve (Cleft Psychology Clinical Excellence Network 2015).
3c. Responsiveness	Clinically meaningful changes in CHASQ scores have not yet been evaluated.
4. Interpretability of scores	Norms have been calculated on 867 CL/P patients from the United Kingdom to aid interpretation of scores from CHASQ. Scores in the 15th percentile indicate lower satisfaction than expected and scores in the 5th percentile indicate very much lower satisfaction than expected (Cleft Psychology Clinical Excellence Network, 2015).
5. Translation of the PROM	Questionnaire developed in English. No other translations avaliable.
6. Patient and investigator burden	Patients can complete CHASQ in approximately five minutes. The level of literacy needed to complete CHASQ has not been evaluated.

CLEFT-Q

CLEFT-Q has been developed with RMT. It consists of 12 scales and one checklist. It has been field tested in 12 countries (Klassen et al. 2018). The CLEFT-Q scales and the User Guide for CLEFT-Q can be found at www.qportfolio.org. Data from Computerised adaptive testing (CAT) has been tested on data from CLEFT-Q (Harrison et al. 2019). The study showed that 97% accuracy of end result could be obtained by using only 43 items (39.2%) of the 110 items in the full scales. An evaluation of CLEFT-Q based on the points from Reeve et al. 2013 can be found in Table 6.

Table 6. Evaluation of CLEFT-Q based on points from Reeve et al. 2013.

1. Conceptual and measurement model	CLEFT-Q is a cleft specific and holistic PROM. It was developed through a process of review of existing PROMs (Klassen et al. 2012), qualitative research with patients with CL/P from six countries (Tsangaris et al. 2017, Wong Riff et al. 2018) as well as field testing in 12 countries (Wong Riff et al. 2017)
2. Reliability	All 12 scales satisfied the requirements for Rasch model analysis. Person separation index values were ≥0.85 for 10 of 12 scales (with extremes) and nine of 12 scales (without extremes). Internal consistency for the 12 scales was tested with Cronbach's alfa (0.89-0.96). Thresholds for response options were checked and corrected when disordered. Item fit statistics, residual correlation, targeting and differential item functioning was evaluated and regarded acceptable (Klassen et al. 2018). Test-retest reliability has not yet been evaluated for CLEFT-Q.
3a. Content validity	Qualitative interviews were carried out in six countries to inform the development of CLEFT-Q (Tsangaris et al. 2017).
3b. Construct validity	CLEFT-Q has shown construct validity since scores varied with cleft type according to severity in line with clinical assessment (Wong Riff et al. 2019). CLEFT-Q scores varied with the need for further intervention as hypothesised (Klassen et al. 2018).
3c. Responsiveness	Clinically meaningful changes in CLEFT-Q scores have not yet been evaluated.
4. Interpretability of scores	Norm values of CLEFT-Q in different groups of age and diagnosis have also been established (Klassen et al. 2018). These norms were based on 2434 children aged 8-29 years from 12 different countries. Norm values for different groups of age and cleft diagnosis were defined as the median score per sample.
5. Translation of the PROM	Documentation is available on method of translation of CLEFT-Q to Swedish and five other languages (Tsangaris et al. 2017, Tsangaris et al. 2018).
6. Patient and investigator burden	Each scale/checklist contains six to 12 items. The answers for each scale are converted using a conversion table to obtain the final score. The administrative burden is considerable if administered in pen-and-paper fashion without the aid of computerised models to calculate the final score. With computerised models and, computerised adaptive testing (CAT), the administrative and patient burden could be much reduced. The time to complete a scale/checklist of CLEFT-Q is only a few minutes for a patient. The level of literacy needed to complete 115 of 199 items in CLEFT-Q is grade 3 level or lower according to Flesch-Kincaid statistics. The psychological and emotional burden inflicted on the patient during completion of CLEFT-Q was investigated during the international field-test of the questionnaire. Patients from 12 countries answered how the instrument had affected them. Of 2047 patients, 88% answered that they liked answering the questionnaire and 12% answered that they didn't like answering it. Of 1922 patients, 25% answered that the questions made them feel better about the way they looked, 72% felt the same way and 3% felt worse after answering the questionnaire. Finally, after answering the questionnaire, of 2048 patients, 77% did not feel upset or unhappy about the way they looked, 20% felt a little upset or unhappy and 3% felt very upset or unhappy (Klassen et al. 2018). In conclusion, a majority of patients were rather happy to fill out the scales and only a few were upset by it.

Aim

The overall aim of this thesis was to investigate PROs in the CL/P population. The perspective of clinical usefulness of implementation of PRO in day-to-day work in the CL/P team permeated all stages of the included studies. This knowledge is expected to allow CL/P teams to better differentiate and prioritize surgical and psychosocial interventions for patients, and in addition, to use PROMs as a tool to enhance patient-clinician communication.

The underlying research questions are listed below. They are applicable to the entire work of the thesis and not specific to a single study. In particular they are relevant to the synthesis of the included five studies and important for development of CL/P care.

a. What do patients themselves think about the outcome of treatment?
b. How can this data be collected?
c. How can this data be used in everyday clinical work and research to develop health care?
d. Which instrument should be used to collect this data?

Specific aims

Study I:

To evaluate satisfaction with speech, perceived speech quality, and perceived improvement from treatment in patients with velopharyngeal dysfunction (VPD).

Study II:

To investigate current beliefs and knowledge of HCPs and parents of children with CL/P in southern Sweden regarding psychosocial and educational issues related to growing up with a visible cleft.

Study III:

To translate CHASQ into eight European languages, to investigate whether levels of PROs in patients with CL/P were comparable within these eight countries and in addition, to investigate clinician experience of the instrument.

Study IV:

To investigate scores of CHASQ in patients with CL/P and a control population. The second aim was to compare CL/P and control population scores with a British norm CL/P population.

Study V:

To investigate the relation of scores between two existing CL/P specific PROMs, CHASQ and CLEFT-Q. The second aim of the study was to investigate patient opinion on the two instruments.

Materials and methods

Different methods have been used in the studies included in this thesis based on the aim of the individual study. Both quantitative, qualitative and mixed methods research have been appropriate on different occasions. Quantitative research design focuses on analysis of numerical data to answer a well-defined, predetermined research question. This method is useful to describe statistical associations or differences between variables (Teddlie and Tashakkori 2009). Qualitative research design focuses on analysis of narrative data in order to answer an often more generic and flexible research question. This method is useful to construct new theories about a studied phenomenon or to describe a process (Nelson 2009). Mixed methods research includes analysis of both numerical and narrative data where both analyses are integrated and contribute to answering the posed research question (Teddlie and Tashakkori 2009).

Specific materials and methods

Study I:

The target group was 222 patients from the southern region of Sweden who had been diagnosed with VPD consecutively examined with videofluoroscopy between September 2002 and May 2010. Videofluoroscopy is a radiographic examination used to visualise the function of the velopharynx. A review of the literature revealed that there was no suitable PROM available for evaluation of VPD. Malmö speech questionnaire, an ad hoc questionnaire was constructed which contained questions concerning patient background, satisfaction with speech, perceived speech quality, and perceived improvement from treatment. A total of 114 participants ultimately returned the questionnaire and were included in the study. Age ranged from seven to 71 years, median age was 14 years. Sixty-seven (59%) of the included patients had CL/P. Patients who had been treated with surgery with or without speech therapy (n=70, 61.4%) and patients who were not referred for surgery and received only speech therapy (n=44, 38,6%) were included in the study. The Mann-Whitney U-test or the Jonckheere-Terpstra test was used for comparing two or more variables. Spearman's test, two-tailed, was used to investigate correlations between variables.

Study II:

An exploratory study design with a qualitative, semi-structured interview methodology was employed. Two groups of interviewees were recruited. In the first group, fifteen parents of children with a visible cleft were interviewed. Inclusion criteria were that the child was nine to 13 years of age and had a visible non-syndromic cleft. The first group comprised nine mothers and six fathers, 37 to 59 years of age. In the second group, 10 interviews were conducted face-to-face with HCPs. HCPs were cleft specialists on the CL/P team, together forming a targeted sample of surgeons, nurses, nurse assistants, orthodontists and SLPs. All were working directly with patients with CL/P and other diagnoses associated with visible differences. Data from this narrative style qualitative study was analysed with thematic analysis.

Study III:

Delegates from participating CL/P centres in Bulgaria, Estonia, Cyprus, Latvia, Macedonia, Romania, Serbia and Sweden translated CHASQ into their respective languages. The translation was carried out according to guidelines from the Mapi Research Trust (2016) for linguistic validation of a PROM. CHASQ was implemented on 220 patients at routine visits to clinics. Age ranged from six to 32 years and mean age was 14.7 years. Differences in CHASQ scores between boys and girls were calculated with Mann-Whitney U Test. Differences in CHASQ scores between countries and diagnoses were investigated using Kruskall-Wallis test. Spearmans's test was used to calculate correlations between non-parametric data. Covariance was analysed with ranked ANCOVA since the residuals in non-ranked ANCOVA were not normally distributed.

Sixteen HCPs involved in the collection of CHASQ data participated in a focus group discussion regarding the strengths and challenges in the use of CHASQ. The discussion was audio recorded and the data analysed in accordance with the principles of thematic analysis (Braun and Clarke 2006).

Study IV:

Data from CHASQ was collected from 64 patients with CL/P (25 girls and 39 boys) on routine visits to the Malmö CLP centre. Age ranged from seven to 19 years; mean age was 12.5 years. The control population consisted of 56 participants (30 girls and 26 boys) who were recruited from a local sports club and a local school. Age ranged from nine to 20 years; mean age was 13 years. Differences in CHASQ scores between the CL/P and control populations were calculated with Mann-Whitney U Test. Differences in CHASQ scores between cleft types were calculated with Kruskal-Wallis Test. Spearmans's test was used to investigate correlations between non-parametric data.

Study V:

Thirty-three patients with CL/P answered both questionnaires CHASQ and CLEFT-Q within the same day or with a couple of days in-between. An extra survey included in the study contained closed- and open-end questions comparing patient opinion of the two instruments. This exploratory part of the study specifically asked for patient opinion about which questionnaire they liked best, which was easier to answer, and which questionnaire they believed would best inform HCPs about patient opinions, feelings and thoughts. Age ranged from 10 to 19 years of age, and mean age was 13. Descriptive statistics was used to examine characteristics of the data. Spearman's correlation was used to test correlations in non-parametric data. Differences in CHASQ and CLEFT-Q scores between subgroups of patients were calculated with Kruskal-Wallis Test.

Results

Summary of specific results

Study I:

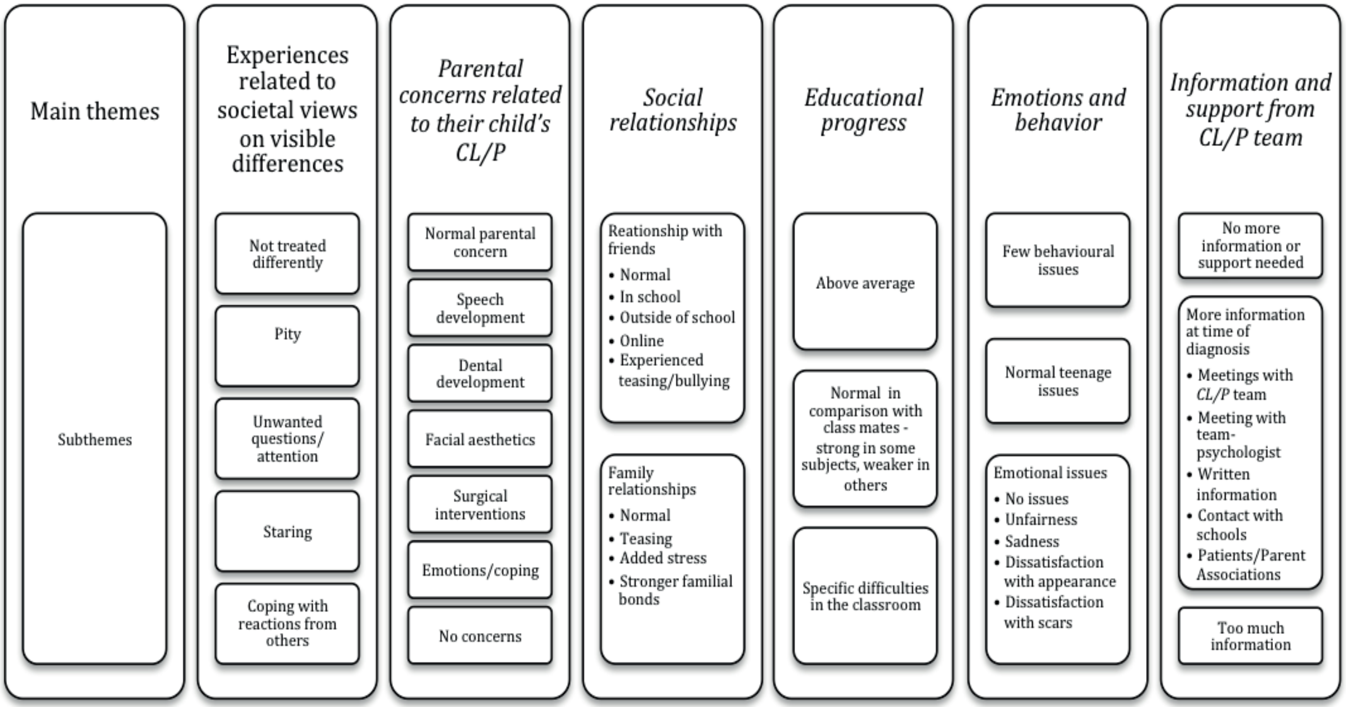
Seventy-one per cent of the patients perceived their speech to be normal or slightly deviant, but only 55% were satisfied with their speech. Sixty per cent of the operated patients felt that the treatment had improved their speech ‘much’ or ‘very much’, 10% thought that they had ‘moderate improvement’, and 30% stated that they had ‘no or little improvement’. From patients who had received only speech therapy, 41% felt that the treatment had improved their speech ‘much’ or ‘very much’, 21% thought that they had ‘moderate improvement’, and 33% stated that they had ‘no or little improvement’. Greater satisfaction with speech correlated with greater perceived improvement from surgery (p=0.005, r=0.350) and with greater perceived improvement from speech therapy (p=0.001, r=0.321). A more normal perception of speech also correlated significantly with greater perceived improvement from surgery (p=0.009, r=0.327) and with greater perceived improvement from speech therapy p=0.005, r=0.283).

Most patients with VPD who underwent evaluation and treatment felt that surgery and speech therapy had improved their speech, but ultimately only approximately 50% were satisfied with the quality of their speech.

Study II:

Parental experience and views were diverse, ranging from the opinion that there were no specific problems related to the cleft - to a clear expression of both emotional and educational issues. The main themes to emerge from thematic analysis were *Experiences related to societal views on visible differences, Parental concerns related to their child’s cleft, Social relationships, Educational progress, Emotions and behaviour, Information and support from cleft team.* Themes and subthemes expressed by parents are presented in Table 7.

Table 7: Main themes and subthemes identified in interviews with parents of patients with CL/P about their views on psychosocial and educational outcomes. The variability of the subthemes reflects the parent's variability of views.



Main themes	Experiences related to societal views on visible differences	*Parental concerns related to their child's CL/P*	*Social relationships*	*Educational progress*	*Emotions and behavior*	*Information and support from CL/P team*
Subthemes	Not treated differently	Normal parental concern	Reationship with friends • Normal • In school • Outside of school • Online • Experienced teasing/bullying	Above average	Few behavioural issues	No more information or support needed
	Pity	Speech development	Family relationships • Normal • Teasing • Added stress • Stronger familial bonds	Normal in comparison with class mates - strong in some subjects, weaker in others	Normal teenage issues	More information at time of diagnosis • Meetings with *CL/P* team • Meeting with team-psychologist • Written information • Contact with schools • Patients/Parent Associations
	Unwanted questions/ attention	Dental development		Specific difficulties in the classroom	Emotional issues • No issues • Unfairness • Sadness • Dissatisfaction with appearance • Dissatisfaction with scars	Too much information
	Staring	Facial aesthetics				
	Coping with reactions from others	Surgical interventions				
		Emotions/coping				
		No concerns				

Regarding social relationships, all parents reported that their child had friends in school and in addition, most children had friends outside of school and/or online. Nonetheless, 13 parents reported that their child was, or had been, teased in school or received unwanted attention related to the cleft and the visible difference. Two parents hypothesised that the family relations had become stronger because of the child having a cleft. On educational progress, 11 parents reported that their children were similar when compared with their peers. Four parents said that their child achieved above average in school. Six parents noted, when asked further, that their child had specific difficulties in the classroom such as concentration, dyslexia, withdrawnness or hyperactivity. All six parents who reported that their child had specific difficulties, however, also reported that their children achieved above average or similarly to their peers in school. Parents reported very few issues in relation to the behaviour of their child. Only one child was reported to have behavioural issues, but no clear diagnosis of these difficulties had been made. Nine children had emotional issues related to their appearance and for eight, this related to their cleft. Seven children were not bothered by their cleft and were satisfied with their appearance. Important information and issues regarding emotional wellbeing and educational achievement were only unveiled after focused discussion on these issues.

The views and level of knowledge of HCPs also varied. The major themes to emerge from the thematic analysis were *Views on visible differences in society*, *Impact of cleft on social functioning, Impact of cleft on psychological functioning, Impact of cleft on educational performance, Factors related to positive adjustment/resilience,* and *Further training needed for HCPs.* Themes and subthemes expressed by HCPs are presented in Table 8.

Table 8: Main themes and subthemes identified in interviews with HCPs about their views on psychosocial and educational outcomes. The variability of the subthemes reflects the HCPs variability of views.

Main themes	*Views on visible differences in society*	*Impact of CL/P on social functioning*	*Impact of CL/P on psychological functioning*	*Impact of CL/P on educational performance*	*Factors related to positive adjustment/ resilience*	*Further training for HCPs*
Subthemes	Increased acceptance • Political correctness • Inclusive culture • Decreased stigma	Possible positive impact not ruled out	No impact on • Cognition • Behaviour • Physical development	No issues	Support from the family	HCPs wanted more information and education
	Possible difficulties • Underestimated intellectual capacity • More attention on appearance in social contexts • Fixation on appearance	Close friendships should not be affected	Challenges • Coping with being different • Bullying • Self-esteem • Shyness • Difficulty in finding friends • Acceptance of themselves • Cognition • Behaviour • Physical development	Issues mainly related to speech and hearing impairment or social impairment	A secure social network	Issues • Lack of time • Low motivation: 'Not my job'
		Negative impact • Children strive to fit in • Bullying • Finding a partner • Few role models • More difficult to make new friends • Parental overprotection		Issues related to *CL/P* diagnosis	Positive attitude	
					Parental income, status, education	
					Parental social competence	

All HCPs wished for more information and training regarding psychosocial and educational issues and treatment. It was however noted by some HCPs that there was a lack of time to address such issues in their clinical work and that they did not want to stigmatize the patients by asking about psychosocial health issues or by suggesting treatment. Neither did they feel that this was 'their job'.

Study III:

CHASQ was translated to eight languages (Bulgarian, Estonian, Greek, Latvian, Macedonian, Romanian, Serbian and Swedish). Kruskal Wallis test for differences between groups of nonparametric data showed a statistically significant difference between countries of score of satisfaction with CL/P related features of the face (p=0,001, χ^2=35.271) and with non-CL/P related features of the face (p=0,007, χ^2=19.248). Results from CHASQ are presented in Table 9.

Table 9. Total score of satisfaction with CL/P related and non-CL/P related features of the face by country.

Country	Number of patients	Mean age in years	Mean score of CL/P related features (max 90)	Mean score of non-CL/P related features (max 60)
Bulgaria	28	15.5	59	53
Cyprus	30	22.9	70	53
Estonia	28	11.9	75	56
Latvia	17	16.0	64	52
Macedonia	30	14.0	61	53
Romania	11	16.3	52	56
Serbia	29	22.2	57	53
Sweden	47	12.4	69	55
Total	220	14.7	65	54

There were patients in each country who were less satisfied than expected according to the CHASQ norm and who should therefore be identified for further investigation or treatment. In the focus group discussion, clinicians in different countries expressed the usefulness of CHASQ in their clinical work. It was perceived as practical, short, and easy to implement. CHASQ could be an effective instrument for collection of PROs on satisfaction with hearing, appearance and speech. Many HCPs using CHASQ in their clinics reported that it helped them gain patient information and informed on treatment decisions. It was used during consultation to spark conversation about patient subjective satisfaction and to suggest alternative pathways of referral.

Study IV:

Children and young people with CL/P were as satisfied with their hearing, appearance and speech as children and young people without CL/P. Higher scores on CHASQ indicated higher satisfaction; 80 points was the highest possible score. Scores of satisfaction with CL/P related features of the face for the CL/P population ranged from 22 to 80 points; mean score was 63. Scores for the control population ranged from 28 to 80 points; mean score was 66. There was no statistically significant difference in satisfaction with CL/P related features of the face between the CL/P and the control population calculated with Mann-Whitney U test (p=0.272, U=2,000.5). Results are presented in Figure 1.

Sixty points was the highest possible score in CHASQ for non-CL/P related features of the face. Scores from the CL/P population ranged from 35 to 60 points; mean score was 55. Scores for the control population ranged from 27 to 60 points; mean score was 52. There was a statistically significant difference in non-CL/P related features between the CL/P and the control population calculated with Mann-Whitney U test (p=0.024, U=1,367.0). The CL/P population had slightly higher satisfaction than the control population. Results are presented in Figure 2. The mean Swedish CHASQ scores of both CL/P and non-CL/P related features of the face were similar to the mean scores in the British norm population with CL/P.

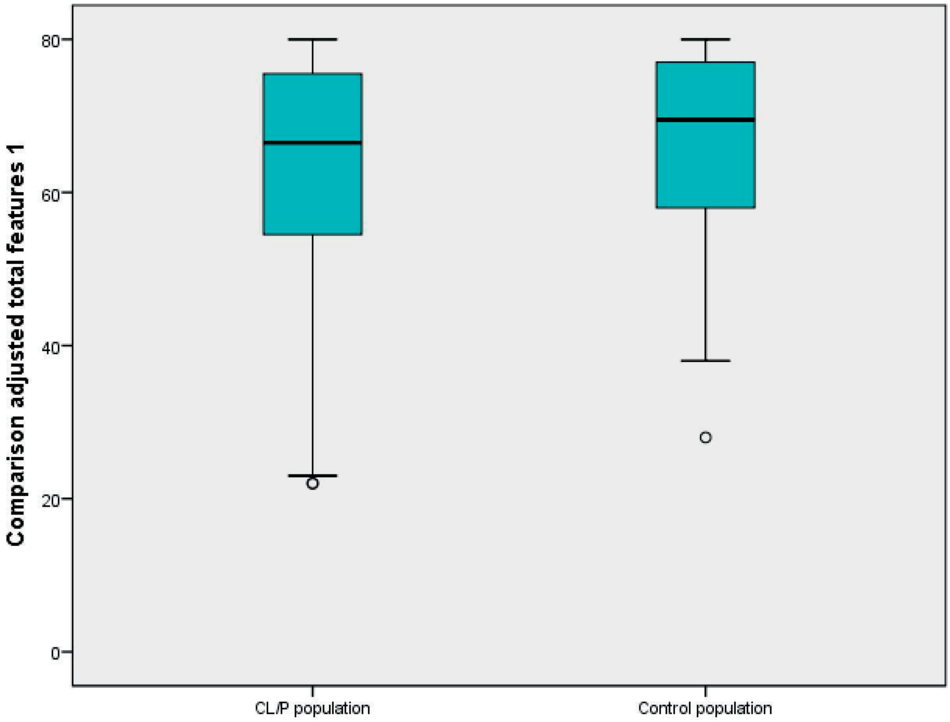


Figure 1. Boxplot showing results of satisfaction with CL/P related features of the face of CL/P population and control population. Median, range, and outliers (º) are presented. High scores indicate high satisfaction.

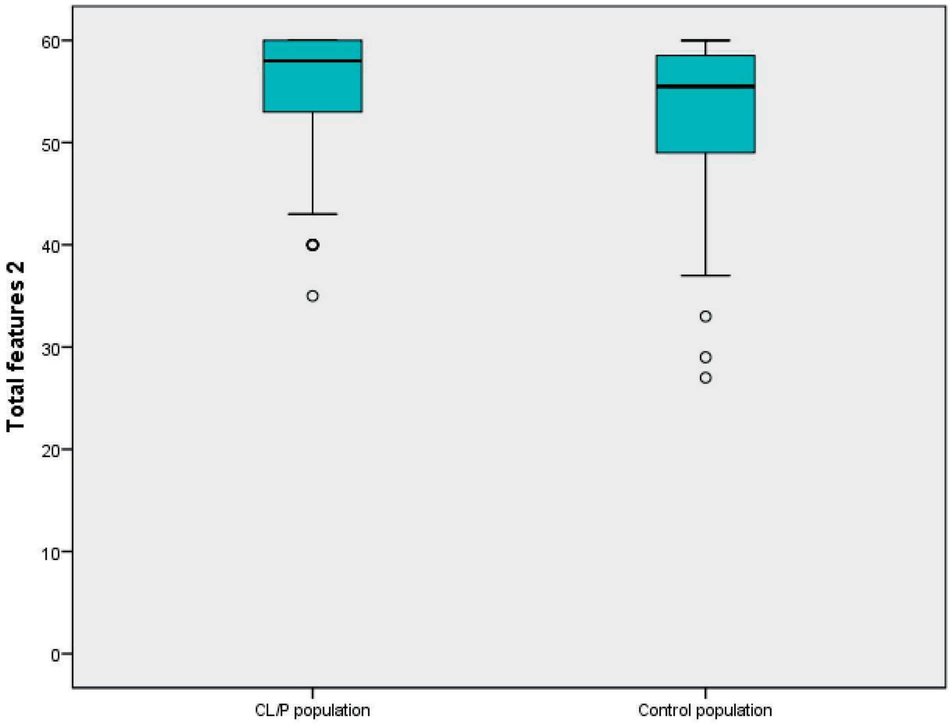


Figure 2. Boxplot showing results of satisfaction with non-CL/P related features of the face of CL/P population and control population. Median, range, and outliers (º) are presented. High scores indicate high satisfaction.

Study V:

Scores from CHASQ and CLEFT-Q correlated well. All items on satisfaction with appearance of the face, specific parts of the face and speech in CHASQ correlated significantly with corresponding scales in CLEFT-Q. Correlations are presented in Table 10.

Table 10. Correlation between CHASQ items and corresponding CLEFT-Q scales calculated with Spearman's correlation.

CHASQ item/CLEFT-Q sclae	p	Spearman's rho
CHASQ Face item – CLEFT-Q Face scale	0.001	0.816
CHASQ Nose item – CLEFT-Q Nose scale	0.001	0.712
CHASQ Nose item – CLEFT-Q Nostrils scale	0.001	0.562
CHASQ Lips item – CLEFT-Q Lips scale	0.001	0.646
CHASQ Lips item – CLEFT-Q Lip scar scale	0.005	0.528
CHASQ Teeth item – CLEFT-Q Teeth scale	0.001	0.735
CHASQ Speech item – CLEFT-Q Speech function scale	0.040	0.413

A majority, 15 patients (58%), who answered the questions in the additional survey, liked CLEFT-Q more than CHASQ. Eighteen patients (69%) thought CHASQ was easier to complete and 19 (76%) that CLEFT-Q would better inform HCPs about their thoughts, opinions and feelings. Descriptive results are found in Table 11.

Table 11. Patient comparison between the CHASQ and the CLEFT-Q

	Total	CLEFT-Q	CHASQ	No difference
Which questionnaire did you like the most?	26	15 (58%)	9 (35%)	2 (8%)
Which questionnaire did you think was easiest to complete?	26	7 (27%)	18 (69%)	1 (4%)
Both questionnaires are meant to help health care professionals understand what you think about yourself and your cleft. Which questionnaire do you think works best?	25	19 (76%)	4 (16%)	2 (8%)

Patients gave various motivations to their preference of questionnaire in the open-ended items in this study. A common theme regarding CLEFT-Q was that they appreciated the depth and detail offered by the more elaborate scales. CLEFT-Q scales regarding feelings and social health were also mentioned as important. Patients who liked CHASQ better seemed to prioritize ease of completion over depth or detail.

Discussion

Importance of PROMs in CL/P care

Results from Study I showed that satisfaction with speech was related to participant perception of speech quality. This is in line with other research showing that self reported satisfaction with facial function and appearance correlates with psychological adjustment and HRQOL (Thomas et al. 1997, Berger and Dalton 2011, Feragen et al. 2017). Other studies have reported that patient satisfaction with speech did not correlate with results from SLPs perceptual speech assessment (Semb et al. 2005, Havstam et al. 2008, Feragen et al. 2017). The same weak association between objective and subjective outcome has also been evident for satisfaction with appearance (Mani et al. 2010, Mani et al. 2013, Pausch et al. 2016). The importance of patient centred care and inclusion of PROs in clinical decisions, audit and research is underscored in Study I and directs attention toward the following studies on PROs in this thesis.

PROMs as an instrument for improved communication between patients and health care professionals (HCPs)

It is understood from interviews with HCPs in Study II, that the feeling of stigmatising the patient is an obstacle for some HCPs when inquiring on psychosocial health. Interviews with parents of patients with CL/P also clearly demonstrated that interviewees had to be asked in detail about education and psychosocial health before they answered that anything might be out of the normal. PROMs have earlier been argued to be able to improve patient-doctor communication (Wormald and Rodrigues 2018) and also to have the potential to facilitate shared decision-making between patients and clinicians (Dobbs et al. 2019).

The testing of CHASQ in eight clinics in Europe, as described in Study III, revealed that clinicians thought that a useful characteristic of CHASQ was the icebreaker function. CHASQ helped HCPs to start talking to patients about their feelings and opinions. Study II clearly illuminated the need for this function. In earlier research, PROMs functioned as an icebreaker during difficult-to-discuss-topics, or when HCPs felt there was a stigma surrounding a topic (Rotenstein et al. 2017). This function of CHASQ can probably be demonstrated by CLEFT-Q as well if implemented in a fashion where the clinician takes time to go through the results together with the patient. It is important for HCPs to review the data and address concerns expressed in results from a PROM. In an earlier study, HCPs noted frustration in their patients if they did not have time to go over the PRO data after patients had taken the time to fill out a PROM (Rotenstein et al. 2017).

Study IV demonstrated that patients appreciated the detail and depth of the scales included in CLEFT-Q. The importance of these characteristics is supported by the results in Study II, since it is possible that patients will not express themselves in such detail spontaneously. CLEFT-Q also includes scales on various domains of psychosocial health, which are precisely the topics where discussion may need to be facilitated due to perceived stigma (Rotenstein et al. 2017).

Generalizability of PROs in patients with CL/P

In Study IV, CHASQ was tested in a control population and showed that the measured constructs were equally affected in young people with and without CL/P. These similar levels of satisfaction are in line with earlier publications on comparisons of SWA scores between CL/P population and control population (Berger and Dalton 2009, Feragen et al. 2015, Crerand et al. 2017). An earlier comparison of Swedish children also showed similar levels of general HRQOL in the CL/P population as in a control population (Sundell et al. 2017). Many scales in CLEFT-Q are too CL/P specific to be tested in a control population. CLEFT-Q scores, however, correlated very well with CHASQ scores in Study V. This finding is in line with earlier comparisons between other PROMs with patients with CL/P (Patrick et al. 2007, Munz et al. 2011, Skirko et al. 2012, Skirko et al. 2013, Broder et al. 2014, Wehby et al. 2014, Ranganathan et al. 2016).

To be able to draw conclusions from either CHASQ or CLEFT-Q about the general level of health within the CL/P population, inclusion of a control population is necessary (Aaronson et al. 2002, Ranganathan et al. 2015, Stock and Feragen 2016, Stock et al. 2018). The results from Study IV and V together demonstrated that the psychological constructs tested in both CHASQ and CLEFT-Q are important to children and adolescents with and without CL/P. The studies also indicated that potential issues in satisfaction with facial appearance or function demonstrated with CLEFT-Q may be within the norm, even though CLEFT-Q was not tested in a control population.

Preferred PROM in CL/P research and clinical work

In the analysis of the three PROMs included in this thesis, CLEFT-Q had higher standards on the points of evaluation presented by Reeve et al. (2013) than CHASQ or Malmö speech questionnaire. In addition, since CLEFT-Q was developed with RMT, it has the potential to be administered electronically with CAT, which would decrease the patient and administrator burden (Harrison et al. 2019).

A majority of patients seemed to prefer CLEFT-Q to CHASQ in Study V because it included questions that were important to them and was more detailed. This may be explained by the fact that the scales and items in CLEFT-Q were generated with patient input through interviews (Tsangaris et al. 2017, Wong Riff et al. 2018). CLEFT-Q is also hypothesized to have higher responsiveness than CHASQ (i.e. to be more sensitive for changes in HRQOL in relation to health care interventions) since it is more detailed (Wormald and Rodrigues 2018).

Implementation of a PROM in CL/P care

A PROM that is perceived as a useful tool in clinical work, and at the same time is valid for research and assesses quality of care in clinical databases, may be easier to implement (Cano et al. 2009). Patients may also more often complete PROMs if they perceive that the questionnaire poses questions that are important to them and their treatment (Cano et al. 2009). Other factors that could affect whether patients feel motivated to complete an entire PROM is the degree of psychological discomfort experienced by the participant and the amount time and effort required (Fitzpatrick et al. 1999, Aaronson et al. 2002).

In Study V a majority of patients thought that CHASQ was easier to complete compared to CLEFT-Q. In Study III, from the perspective of usefulness in day-to-day clinical work, CHASQ was also assessed as an easy to use and brief instrument. CHASQ can therefore be assumed to be easier to implement than CLEFT-Q. However, patients in Study V seemed to like CLEFT-Q and think that it was a better way to inform HCPs (i.e. CLEFT-Q had high face validity). It may therefore be completed by patients to a greater extent (Cano et al. 2009).

There may be some reluctance to research patient HRQOL and implement PROMs since HCPs may not be prepared to meet strong emotions, sadness or critique from patients or may feel that it is not ‘their job’ (Cadogan 2012). This issue, which became evident in Study II, can be addressed before implementing a PROM. Fears and insecurities in HCPs can be counteracted with appropriate information, education and outline of a clear pathway of referral for patients in need of further investigation or psychological treatment (Clarke and Cooper 2001, Stock et al. 2019).

In earlier studies on implementation of PROMs, challenges have been described such as lack of time, higher costs, having to change clinic infrastructure, having to sift through large amounts of data, detailed scoring systems and difficulty in accessing data in electrical medical records (Hutchings and Alrubaiy 2017, Rotenstein et al. 2017, Kaur et al. 2018). In general, however, HCPs felt that PRO-data was valuable to them, made them aware of additional symptoms and patient satisfaction, and gave them a starting point to talk about difficult-to-discuss symptoms (Rotenstein et al. 2017, Kaur et al. 2018). One further challenge in the implementation process is that PRO and objective outcomes may be considered as two competing sciences. If so, perceived incoherence between PRO and objective outcomes may undermine the credibility of PRO. For example, clinicians may think that results from an objective outcome are correct and that PROs that do not match them therefor must be incorrect. This scenario is unfortunate, since PRO and many kinds of objective outcomes are complementary in treatment (Brook et al. 2017). Conceptualizing a research topic too broadly, as for example appearance satisfaction, without specifying which population, age group or which construct within appearance satisfaction is being targeted, has been noted as a methodological issue (Krawczyk et al. 2012). This may contribute to a perceived inconsistency between studies of different constructs and populations showing various study results.

Strengths and limitations

Ideally, greater focus should be directed to cross-disciplinary research and to include a wide range of perspectives in order to identify and link up new ideas within, for example neuroimaging, objective aesthetic outcome, speech assessment, facial function, PRO, psychosocial health, educational outcomes, as well as sociocultural circumstances (Haidt 2006, Nelson 2009, Thompson 2012, Stock et al. 2018). Issues related to the narrow scope in studies on HRQOL in the CL/P population have been discussed earlier (Feragen et al. 2015, Feragen and Stock 2016, Stock et al. 2016). From this perspective, the overall limitation that applies to the studies included in this thesis is the narrowness and specificity of the method and analysis. In Study III, for example, the addition of an objective rating of surgical aesthetic results or socioeconomic status would have added scientific value since the significant differences in CHASQ score could not be explained by variables collected in the study.

Collaborative studies between different centres and countries are beneficial and can teach us more about PROs than separate individual efforts. It is an often-mentioned problem within this field of research that varying research methodology and the use of ad hoc PROMs, make results from different studies difficult to compare and to build upon (Cano et al. 2004, Eckstein et al. 2011, Klassen et al. 2012, Feragen et al. 2014, Stock and Feragen 2016). Results obtained from Study I were collected with an ad hoc PROM that meets the standards of few points of evaluation of the quality of a PROM (Reeve et al. 2013). This study design is a limitation to the quality of the PROs gathered in Study I. At the time of the study, however, no suitable PROM that included satisfaction with speech related to VPI or improvement after treatment existed (Eckstein et al. 2011). Therefore, to answer the research question, an ad hoc PROM was created. Since the publication of Study I, VELO, a disease specific PROM developed for evaluation of VPD has been published (Skirko et al. 2012, Skirko et al. 2013).

It is a strength of this thesis that the authors of the included studies work independently from any of the teams of researchers who developed CHASQ or CLEFT-Q and would not benefit financially by favouring any instrument.

Specific limitations and strengths regarding methodology per study are listed in Table 12.

Table 12. Limitations and strengths specific to each study.

Study	Limitations	Strengths
I	No evidence of reliability or validity of the PROM in CL/P population. Qualitative method not included. Heterogenous study population regarding age and diagnosis.	The study included all patients who underwent videofluoroscopy, including patient groups who often are excluded from studies.
II	No patient perspective. No school records.	The study highlighted information on and beliefs about psychosocial and educational outcomes that would have easily been missed with a quantitative method approach.
III	No objective aesthetic or perceputal speech assessment. No socioeconomic variables. Convienence sample of study participants.	The mixed method approach highlighted important perspective on positive qualities of implementing CHASQ. The multinationality of the study makes results easily extrapolated to different clinics and settings.
IV	No aesthetic or speech assessment by HCPs. No socioeconomic variables. Qualitative method not included.	The study added interpretability of scores from CHASQ in the CL/P population.
V	No aesthetic or speech assessment by HCPs. No socioeconomic variables. Small research population.	The study included quantitative and qualitative patient perspective on preferred PROM for implementation. Authors were independent from teams who developed the included instruments.

Future considerations/directions

Background variables such as socioeconomic status of participants should be considered in HRQOL research since income and educational level has been shown to affect mental health (Reiss 2013). There are also indications that individuals with CL/P, compared to otherwise healthy control populations, have a higher proportion of additional conditions (such as developmental delay, learning difficulties, autism spectrum disorders or ADHD) (Feragen et al. 2014, Tillman et al. 2018), negatively affected education (Persson et al. 2012, Wehby et al. 2014, Knight et al. 2015, Watkins et al. 2018) and private economy (Marcusson et al. 2001). A population based Norwegian study, however, found that socioeconomic status was not affected for participants with CL/P (Berg et al. 2016). As discussed by Feragen and Borge (2010) earlier, the impact of socioeconomic status on other study results may also be lower in Norway than in other societies, since education and health care is free. Since education and health care is also free in Sweden, the same may be assumed for studies on Swedish participants.

Mixed methods research should be applied when possible in research on PROs to validate the chosen quantitative methodology in complex research questions (Teddlie and Tashakkori 2009, Dures 2012, Regnault et al. 2017). The risk for overlooked processes and faulty assumptions of causality is otherwise high in a research field such as HRQOL, where the patient population is stretched over a wide range of age, undergoing various developmental stages and interventions in multiple specialities (Nelson 2009, Sharif et al. 2013). Qualitative methods also assist in understanding differing or contradictive quantitative results from, for example, HCPs, laypersons as well as a patients (Nelson 2009).

Small research populations are a common issue at present due to low prevalence of CL/P and stratification into subgroups (Stock et al. 2018, Stock and Feragen 2019). This poses a methodological challenge that makes qualitative research suitable for learning more about the CL/P population (Nelson 2009, Stock and Feragen 2019). To compensate for small research populations, various HRQOL outcomes could be reported on a national basis to a quality registry. This would ensure both multi-centre data and study populations large enough for multivariate statistical tests and subgroup stratification (Persson 2012, Stock et al. 2016).

Conclusions

The results presented in this thesis indicate that future efforts in care and research in this patient group should include implementation of a CL/P specific and holistic PROM. Consensus on a nation wide level and adaption of the same PROM and method for implementation is favourable. The overall conclusions from the work included in this thesis are listed below.

a. PROs scores in the CL/P population are generally high and similar to those of a control population in regard to satisfaction with hearing, appearance and speech.

b. Patient and family reported outcome and opinion can be collected with PROMs, interviews and focus group discussions.

c. A PROM can be a useful instrument for enhanced communication between clinicians and patients and their families. PROs have a central role in the development of patient centred care.

d. A CL/P specific, holistic, validated and internationally accepted PROM should be implemented. At the time of writing, CLEFT-Q is the most appropriate PROM.

Summary in Swedish Populärvetenskaplig sammanfattning på svenska

Läpp-käk-gomspalt (LKG) är en av de vanligaste medfödda avvikelserna. Cirka två av 1000 barn föds med någon typ av LKG. I Sverige behandlas barnen vid regionala centra med flera olika specialiserade vårdgivare till exempel plastikkirurg, logoped, öron-näsa-halsläkare, ortodontist, psykolog, kurator, kontaktsköterska och omvårdnadspersonal. Behandlingen innebär olika kombinationer av operationer, logopedisk utredning och behandling, tandreglering och därutöver sjukhusbesök för uppföljning från det att barnet är nyfött och ända upp till vuxen ålder.

Olika aspekter av barnens och ungdomarnas hälsorelaterade livskvalitet (HRQOL) kan påverkas av LKG och dess behandling. HRQOL definieras enligt internationell standard som ”den funktionella effekten av ett medicinskt tillstånd och/eller dess behandling på en patient”. HRQOL är alltså subjektivt och flerdimensionellt, omfattande fysisk och yrkesmässig funktion, psykologiskt tillstånd, social interaktion och somatisk känsla”. Hög HRQOL är ett av de viktigaste målen i behandlingen av LKG.

Patientrapporterade utfallsmått (Eng: Patient Reported Outcome Measures, PROMs) mäter olika aspekter av HRQOL ur patienternas perspektiv. Eftersom att många plastikkirurgiska interventioner fokuserar på att öka livskvaliteten och patientens självuppfattning är patientens perspektiv central för att utvärdera resultaten av behandlingen. PROMs har också potential att leda till förbättrad kostnadseffektivitet inom plastikkirurgi, förbättra kommunikationen mellan patient och läkare och, i ett längre perspektiv, förbättra patientnöjdheten. Det övergripande syftet med denna avhandling var att undersöka patientrapporterade utfall (Eng: Patient Reported Outcomes, PROs) i patientgruppen med LKG. Det kliniska perspektivet och användbarheten av PROMs i det dagliga arbetet i LKG-teamet genomsyrar alla studier i avhandlingen.

I Studie I utvärderades nöjdheten med tal, upplevd talkvalitet och upplevd förbättring av kirurgisk och/eller logopedisk behandling hos patienter med velopharyngeal dysfunktion (VPD). VPD innebär svårighet att stänga passagen mellan munhålan och näshåla med hjälp av mjuka gommen (velum) och svalgväggarna (farynx). Resultaten visade att de flesta patienterna med VPD ansåg att kirurgi och/eller talträning hade förbättrat deras tal, men bara ungefär hälften av dem var i slutändan nöjda med sin egen talkvalitet.

I Studie II var målet att undersöka uppfattningen och kunskapen om psykosociala frågor och skolgång hos barn och ungdomar med LKG. Intervjuer genomfördes med föräldrar till barn med LKG i södra Sverige och med sjukvårdspersonal. Föräldrarnas erfarenhet och åsikter var varierande, allt från att det inte fanns några specifika problem relaterade till LKG till tydliga problem av både emotionell karaktär och angående skolgången. Åsikterna och kunskapsnivån hos sjukvårdspersonalen varierade också. All personal önskade mer information och utbildning i psykosociala frågor och om utbildning i LKG-gruppen. Denna studie visade också att viktig information rörande emotionell hälsa och skolgång först uppdagades efter fokuserad diskussion om dessa frågor.

I Studie III översattes Cleft Hearing Appearance and Speech Questionnaire (CHASQ) till åtta europeiska språk: bulgariska, estniska, grekiska, lettiska, makedonska, rumänska, serbiska och svenska. PROs från de olika länderna jämfördes och sjukvårdspersonals uppfattningar om instrumentet undersöktes. Studien visade på olika nivåer av patientnöjdhet bland de olika länderna. Det fanns patienter i alla länder som enligt sin poäng på CHASQ var missnöjda och borde identifieras för vidare utredning eller behandling. I en fokusgruppsdiskussion uttryckte sjukvårdspersonal i många länder att CHASQ uppfattades som kort och lätt att använda. CHASQ kan således vara ett användbart instrument för att samla in PRO om nöjdhet med hörsel, utseende och tal.

I Studie IV undersöktes om det fanns några skillnader i poäng på CHASQ mellan patienter med LKG och en kontrollgrupp med barn och ungdomar utan LKG. Det andra syftet var att jämföra LKG- och kontrollgruppens resultat med en brittisk grupp av patienter med LKG. Barn och ungdomar med LKG i denna studie var lika nöjda med sitt utseende, hörsel och tal som barn och ungdomar utan LKG. Poängen på CHASQ var i samma nivå med den som uppmättes i den brittiska LKG-gruppen.

I Studie V var syftet att undersöka förhållandet mellan poängen på två befintliga LKG-specifika PROMs, nämligen CHASQ och CLEFT-Q. Syftet var också att undersöka vad patienterna tyckte om de två instrumenten efter att ha besvarat båda. Poängen på CHASQ och CLEFT-Q korrelerade väl. En majoritet av deltagarna tyckte att CHASQ var lättare att besvara än CLEFT-Q. En majoritet av deltagarna föredrog dock CLEFT-Q och ansåg att den hade bättre potential att uppmärksamma sjukvårdpersonal på deras känslor och tankar jämfört med CHASQ.

Resultaten från denna avhandling visar på att framtida utveckling av vården inom denna patientgrupp bör omfatta implementering av ett PROM som är LKG-specifikt och holistiskt, det vill säga som omfattar många perspektiv av HRQOL. Att nå konsensus på nationell nivå kring vilken PROM och vilka implementeringsmetoder som bör användas är en förutsättning för att resultat ska kunna jämföras mellan centra.

Sammanfattningsvis är PROs inom LKG-gruppen generellt höga och i samma nivå som i en kontrollgrupp vad gäller nöjdhet med hörsel, utseende och tal. Patientens och deras familjemedlemmars egenrapporterade resultat och åsikter kan samlas in med PROMs, intervjuer och fokusgruppsdiskussioner. PROMs kan vara användbara instrument för förbättrad kommunikation mellan sjukvårdspersonal och patienter och deras familjer. PROs har en central roll i utvecklingen av patientcentrerad vård. En LKG-specifik, holistisk, validerad och internationellt accepterad PROM bör implementeras. I nuläget är CLEFT-Q den bäst lämpade PROM.

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