OBSERVATION

PREFACE

The Observation section of this issue contains two papers that take different approaches to the same topic, namely directions for future research. The first paper by Morris and Bardach is actually a summary of the proceedings of a large 'State of the Art' meeting held last year in Iowa City. Having attended that meeting, I know that their task of compiling this manuscript was a formidable one and represents a distillation of over 120 separate opinions regarding where we, the readership, might best direct our efforts in the future. Mr. McComb’s paper is a personal essay on his own perception of the direction future research should take, and it represents the remarks of someone with the benefit of a long and distinguished career. The reader will find that the underlying messages of these two papers are remarkably consistent. For this reason, they have been grouped together in this issue.

The Editor

Cleft Lip and Palate and Related Disorders: Issues for Future Research of High Priority

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On October 14 to 17, 1987, a meeting entitled "State of the Art Conference: Multidisciplinary Management of Cleft Lip and Palate" was held in Iowa City. The major purpose of the conference was to review the available knowledge concerning management of unilateral cleft lip and palate from the perspectives of surgery, speech-language pathology, and orthodontics. The closing feature of the conference was the identification of issues for future research. Participants identified issues of high priority that were then discussed by the entire conference faculty. This paper reports the summary of that discussion and the recommendations of the Conference.

KEY WORDS: cleft lip and palate, multidisciplinary management, prevention, maxillofacial growth, psychosocial, nasal airway obstruction, velopharyngeal insufficiency

In 1987, from October 14 through October 17, a meeting was held in Iowa City entitled "State of the Art Conference: Multidisciplinary Management of Unilateral Cleft Lip and Palate." The purpose of the conference was to review the available knowledge concerning management of unilateral cleft lip and palate from the perspectives of surgery, speech-language pathology, and orthodontics. However, during the discussions, frequent reference was made to other cleft types and other craniofacial disorders. The role of other disciplines was also discussed. There were 121 participants from 20 states and 22 countries invited, based on major achievements and contributions to their disciplines. The conference was supported by the Department of Otolaryngology–Head and Neck Surgery, gifts received by the University of Iowa Foundation in support of cleft palate research, individual registrations, and the National Institute of Dental Research.

One goal of the conference was the identification of high priority issues for future research. Participants identified these issues individually, and on the final day of the conference, three participants (Betty Jane McWilliams, Howard Aduss, and Peter Randall) led discussions to delineate them further. The material generated from that discussion serves as the basis of this report. The first draft of this report was later reviewed by 15 of the participants for further review and commentary.

ISSUES OF HIGH PRIORITY

Prevention of Clefts

Various approaches for the prevention of clefts by the administration of therapeutic agents to high-risk mothers have been advocated, but none has yet proven effective. However, the concept of prevention must be given the highest priority. Centers with large clinical populations are particularly well suited to pursue these investigations.
Embryology

Noninvasive methods that will allow a better description of embryogenesis are now available or are in the process of being developed. These methods can be expected to provide important information about the process of dysmorphogenetic processes that contribute to clefting. An outgrowth of these investigations would be the facilitation of early prenatal detection. However, the ethical and legal implications for prenatal detection must be considered and explored.

Growth and Development

The interactions between a structural birth defect such as cleft lip and palate and the growth and development of the individual must be considered in multidisciplinary treatment planning. The two following specific aspects of growth and development were targeted as having special importance: maxillofacial growth and speech and language development. These two areas are closely linked because of the large number of modalities that have been advocated for the treatment of palatal clefts. We must understand how the cleft defect itself affects growth and development, while simultaneously exploring the effects of the treatments applied to the cleft, including the psychosocial variables of our clinical efforts.

Specific Issues Relating to Growth and Development

The issue of maxillofacial growth and development was divided into five specific areas for investigation. It should be pointed out that some participants thought that this area should include “craniofacial” issues as well as “maxillofacial.” The five specific areas include the following:

1. Patterns and mechanisms of normal and abnormal growth and development of both skeletal structures and soft tissues.
2. The identification of factors that enhance or inhibit bone formation and growth.
3. Criteria for determining parameters of normal and abnormal development (intrinsic and extrinsic factors). Both deficiency and excess as dysmorphologic processes should be investigated.
4. The development of models (animal or computerized) for predicting growth and development patterns.
5. The effect of untreated cleft lip and palate on growth and development.

The issue of speech and language development was divided into four specific areas of priority, as follows:

1. Patterns and mechanisms of both normal and abnormal development.
2. The identification of factors that enhance or inhibit normal speech and language development, such as genetics, the environment, communicative efficiency, personality factors, and the relation of speech and language to other sensory processes.
3. The development of models (computer, theoretical, or others) for predicting developmental patterns.
4. The effect of untreated clefts on speech and language development.

Treatment Issues

Although there has been an extensive literature concerning the effects of treatment on cleft lip and palate, there are still many areas of contention. Recently developed methods require careful scrutiny, while more familiar methods merit more careful study. There is a special need for data from consecutive series of large numbers of subjects that reflect comprehensive evaluation by many disciplines, using a carefully described management regime. Research designs must deal with the heterogeneity of the defect. There is a special need for a continued focus on surgical intervention, because surgical results largely determine the extent of subsequent needs (i.e., health, function, appearance, and psychosocial status). The clinical efficacy of treatments must be determined so that professionals and patients can better evaluate the relative merits of various strategies.

Specific Treatment Issues: Surgery

The criteria for intervention with various techniques should be determined as they relate to the severity of the defect, aesthetics, speech-language status, maxillofacial growth, and psychosocial status. Practical considerations, such as the availability of resources, must also be considered.

The results of different types of surgical interventions must be assessed from many perspectives. Factors such as surgical expertise, technique, timing, and biologic responses to the healing process should be included in such research. Results should be analyzed in a multidisciplinary manner.

Specific Treatment Issues: Speech and Language

Criteria for intervention must be developed. Factors such as general health, age, developmental status, socioeconomic status, psychosocial status, family expectations, and availability of resources must be considered. The morphologic, neurologic, and behavioral mechanisms affecting the development and maintenance of speech and language skills should be identified.

The determination of the effect of various surgical procedures on speech development is perhaps of foremost importance. This research should include a careful assessment of technique, timing, and efficacy.

Specific Treatment Issues: Orthodontic Intervention

The criteria for intervention should be determined and should take into account dental status, maxillofacial development, health, developmental status of the child, family expectations, the availability of resources (including funding), and age. The biologic mechanisms affecting orthodontic management and their influence on subsequent function and appearance should be delineated.

The differential results of various types of orthodontic treatment should be determined. This research should include relation to surgery (both technique and timing), speech (particularly articulation), and skill levels.
Other Treatment Issues

The importance of appropriate pediatric care, feeding techniques, otolaryngologic care, genetic counseling, patient and family counseling, prosthetic dental procedures, and other procedures also requires further study, especially as these treatments relate to complete multidisciplinary management.

Clinical Efficacy of Treatments

The identification and assessment of cost-benefit factors in health care delivery of all kinds are expected to address “adequacy” and “effectiveness” as well as “efficacy” of treatment. These terms have specific operational definitions and are usually studied by a clinical epidemiologist in close collaboration with clinicians (assisted by biostatisticians and others). The evaluation of the “utility” of alternative clinical strategies is the standard for determining how useful a treatment is in the context of clinimetrics and clinical decision analysis. Data about clinical efficacy of treatment come from a variety of sources. Some data come from investigative efforts by the traditional delineation of separate clinical specialties, which collectively represent the team approach. Another promising source of data is clinical trials by multiple centers, using different treatment approaches, but the same inclusion and exclusion criteria.

Specific Issues Related to Research Design

There have been many different types of research designs used to determine clinical efficacy. The following factors will need to be resolved within each type of research design:

1. Retrospective designs
   - availability of suitable data
   - reliability of data
   - identification of sources of bias
   - applicability or generalization of the findings

2. Prospective designs
   - ethical considerations
   - definition of criteria for entry into the study (inclusionary and exclusionary criteria)
   - availability of suitable subjects for the study
   - practicality (e.g., compliance, cost)
   - consistency of methodology (the avoidance of midstream changes in protocol or philosophy)

3. Single center studies
   - applicability of methods and findings to other centers
   - cross-sectional studies
   - longitudinal studies

4. Multicenter studies
   - agreement about consistency and criteria between centers
   - advantages and disadvantages of pooling subjects
   - cross-sectional studies
   - longitudinal studies

5. Randomized trials
   - criteria for assignment to treatment groups
   - ethical considerations of random assignment to treatment groups
   - informed consent for participation

6. Outcome assessment
   - criteria for success and failure.

Psychosocial Aspects

Psychosocial research must not only focus on the adult following treatment, but also on all age levels during the treatment process. The family of affected individuals must also be studied. Two important research design problems are the multivariate nature of psychosocial status and the identification of reliable and valid methods for assessment. The following issues require extensive study: the identification of parameters of importance (such as family acceptance, self image, educational achievement, acceptance by peers); methods of assessment; developmental perspectives; academic and educational implications; relationships to communication skills, aesthetics, treatment, and perceptions about etiology; special problems in adolescence and adulthood; and methods for prevention of psychosocial problems.

Velopharyngeal Mechanism and Function

Cleft palate speech is distinctive because of the combination of disorders of resonance and articulation. Although hypernasality is the more obvious problem associated with clefting, hyponasality may also occur secondary to nasal obstruction, pharyngeal flap, or excessive obturation with a prosthesis. The accurate diagnosis of velopharyngeal function is crucial to the treatment of speech disorders in patients with clefts.

Specific Issues for Research

The following issues require further delineation and study:

1. A working definition of clinically significant velopharyngeal dysfunction.
2. The validity, reliability, practicality, cost effectiveness, and compliance factors associated with methods of assessment.
3. Developmental aspects of velopharyngeal function.
4. The stability of velopharyngeal function and factors that might affect that stability.
5. The relationship between velopharyngeal function and resonance characteristics.
6. The outcome of various types of physical management of velopharyngeal dysfunction with special reference to the timing and technique of primary palate repair.
7. The effect of fistulae, unrepaired anterior palatal clefts, or both on velopharyngeal function on short- and long-term bases.
8. The efficacy of various treatment modalities in the treatment of velopharyngeal disorders.

Nasal Airway

Abnormalities of the nasal septum and the surrounding nasal structures may result in a restricted nasal airway in individuals with clefts of the lip and palate. Nasal airway obstruction may also be caused by iatrogenic effects (such as pharyngeal flaps) or hypertrophy of the lymphoid tissues.
Issues requiring study in some respects are similar to others mentioned above. Definitions of nasal obstruction, methods of assessment, and developmental aspects of nasal and oral respiration must all be delineated. But beyond these issues, the following aspects are specific to nasal airway problems:

1. The relationship between nasal and oral respiration patterns and subsequent growth and development (including facial skeletal development).
2. The relationship between nasal and oral respiration and the resonance characteristics of speech.
3. The relationship between nasal and oral respiration and the anomalies associated with clefting, both before and after surgery.
4. The relationship between nasal and oral respiration and general health.

COMMENTS ABOUT IDENTIFIED PRIORITIES

As expected, there was considerable variation among the participants in the identification of high priority issues. Some issues were very specific in nature, such as identifying the components of the resonance characteristics of speech or the induction of bone growth in cleft sites by electric current. Others were more general, such as the need to study the timing of primary palatoplasty in relation to both speech development and midfacial growth. Although diversity was apparent in this group, there was a consensus about certain generic priorities that cut across all of the areas of study cited above, as follows:

1. The prevention of cleft lip and palate and related disorders.
2. The identification of the most effective primary management techniques to minimize the need for secondary procedures for speech, aesthetics, malocclusion, and oral function.
3. Improved ability to predict problem cases so that their early identification can lead to more effective management.
4. Continued evaluation of individual treatments and treatment systems.
5. The identification of better methods of secondary management for more effective salvaging of problem cases.
7. Continued attention to the patient and family so that personal development is hampered as little as possible by the cleft.