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# Memoranda/Memorandums

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## Implementation of cystic fibrosis services in developing countries: Memorandum from a Joint WHO/ICF(M)A meeting\*

*A Joint WHO/ICF(M)A meeting defined strategies for the implementation and development of cystic fibrosis (CF) services in countries where CF is thought to be more common than is appreciated by the medical profession or the public and where CF services are either non-existent or in the early stages of development. The following common problems in such countries were identified: underdiagnosis; reduced life expectancy (relative to developed countries) among those diagnosed; a clinical profile that may vary from the "classic" description; poor availability of necessary drugs; and a lack of CF services or basic research on the condition.*

*The following recommendations were made concerning CF: neonatal screening projects should be set up to determine its incidence and identify affected infants; national and regional laboratories should be encouraged to identify the specific mutations responsible for the condition in their populations; centres for diagnosis and treatment should be developed, using a team approach; a national organization, involving families and friends of individuals with CF and other volunteers, should be established; a national registry should be set up; awareness about the condition should be improved and knowledge about it developed among medical professionals, public health authorities, and the general public; and increased collaboration between groups and organizations (including pharmaceutical companies) at the national, regional, and international levels should be developed in order to exchange information about and promote knowledge of CF, and to stimulate the development of CF services in developing countries.*

### Introduction

Cystic fibrosis (CF) was first recognized about 60 years ago in Europe and the USA. In early descriptions, life expectancy was generally less than 2 years. Diagnosis was based on pancreatic malabsorp-

tion accompanied by chronic pulmonary infection. Family histories demonstrated that the inheritance pattern was autosomal recessive. With the development of sweat testing in the 1950s, diagnosis became easier and more accurate, and was made with increasing frequency. CF is now recognized to be the commonest serious single-gene disorder in most Caucasian populations.

With the treatment available in specialized centres, including attention to all major features of the disease (respiratory, digestive, etc.), the prognosis in many countries has improved. Mean actuarial life expectancy is now about 30 years. However, such figures are historical; prospective estimates for young children with CF suggest that they may live for 40 years or more, even without the development of new treatment modalities. Since the identification in 1989 of the gene defect causing CF, there has been unprecedented progress in the understanding of its mechanisms and in new approaches to the development of definitive pharmacological therapy. Such treatments are expected to be available within the lifetime of most patients alive today.

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\* This Memorandum is based on the report of a Joint WHO/International Cystic Fibrosis (Mucoviscidosis) Association (ICF(M)A) Meeting held in Manama, Bahrain, 18–19 November 1995 (unpublished document WHO/HGN/ICF(M)A/WG/95.6, available upon request from Division of Noncommunicable Diseases, World Health Organization, 1211 Geneva 27, Switzerland).

Participants at the meeting were as follows: Dr S. Al-Arrayed, Manama, Bahrain; Dr F. Al-Mahroos, Manama, Bahrain; Dr G. Al-Thani, Doha, Qatar (*Rapporteur*); Professor G.J. Barbero, Columbia, MI, USA; Professor J.A. Dodge, Belfast, United Kingdom (*Chairman*); Dr C.N. Macri, Buenos Aires, Argentina; Dr H. Nazer, Riyadh, Saudi Arabia; Dr I. Popa, Timișoara, Romania; Dr M. Rawashdeh, Irbid, Jordan; Dr G. Ruggeri, Caracas, Venezuela. *ICF(M)A Secretariat*: Mrs L. Heidet; Mr M. Weibel. *WHO Secretariat*: Dr V. Boulyjenkov (*Secretary*).

Requests for reprints should be sent to Dr V. Boulyjenkov, Human Genetics, Division of Noncommunicable Diseases, World Health Organization, 1211 Geneva 27, Switzerland.

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Previous joint meetings between WHO and the International Cystic Fibrosis (Mucoviscidosis) Association (ICF(M)A) have considered the geographical distribution of CF and its control, as well as population screening and current and prospective treatment (1-3). Although CF is widespread, there are significant variations in its incidence, with relative sparing of African and Asian races. Recognition of CF, and the provision of appropriate clinical services, are generally good in western Europe, the USA, and Australasia. However, there are other large populations in whom the disease is infrequently recognized and inadequately treated, resulting in avoidable death or suffering in infancy, childhood, and adulthood. The Joint WHO/ICF(M)A meeting reported here was convened to address these problems and to determine strategies for the implementation and development of CF services in developing countries. In this context, "developing country" refers to a country where CF is known to exist, where it is thought to be much more common than is appreciated by either the medical profession or the general public, and where CF services are either non-existent or in the early stages of development.

## Cystic fibrosis in developing countries

Participants at the meeting were asked to complete questionnaires on the overall conditions of and problems regarding the availability of CF care in their countries. The following common problems were identified:

- underdiagnosis of CF;
- reduced life expectancy (relative to developed countries) among those diagnosed with CF;
- a clinical profile that may vary from the "classic" description of CF;
- poor availability of necessary drugs; and
- a lack of CF services or basic research on CF.

Against this background, the objectives of CF services in developing countries should be to improve diagnosis and survival and to define the clinical profile of CF in the local population, as well as to explore possibilities for improving the supply of appropriate medications.

The availability of health services and the level of socioeconomic development varied considerably among the countries represented. Although the relative contribution of the public and private health care systems to CF services was not discussed, the

principle of the right to appropriate care for all individuals with CF was assumed.

## Prerequisites for establishing a cystic fibrosis service

**Case-finding.** Simple tools must be available for identifying affected individuals. The essential diagnostic tool is the sweat test, in which sweating is induced by the local application of pilocarpine by means of iontophoresis. The sweat is collected under standardized conditions and its sodium chloride content is analysed. The test must be carried out meticulously by trained and experienced personnel. Although some commercial adaptations have been developed for use outside specialized laboratories, they should be regarded only as screening tests and, when positive, the individual should be referred to a centre able to carry out a definitive test before diagnosis is confirmed.

Neonatal screening offers another possibility for diagnosis and may be of particular value in estimating the burden of CF in a population where it is underdiagnosed. There are two methods of neonatal screening: the measurement of immunoreactive trypsin in dried blood spots and the measurement of the albumin level in meconium using a test strip. The latter is cheaper but has a false-negative rate of about 15%. Nevertheless, in populations such as those in Latin America, where it is estimated that 90% of affected individuals die without a diagnosis having been made, it offers the possibility of improved screening at relatively low cost. Both immunoreactive trypsin and meconium testing are strictly screening tests, and diagnosis cannot be made until a definitive sweat test or genetic analysis has been performed.

**Medical staff.** A minimum of one or two dedicated physicians with an interest in CF can provide the starting point for a diagnostic and clinical service for the condition. It is essential for these physicians to be highly motivated and willing to provide leadership. Although it is helpful, it is not essential for them to work in the capital or a large city with access to the resources of a major hospital.

**Training facilities.** The care of CF patients requires a team of professionals, all of whom need training and the opportunity to develop experience. The necessary skills must then be passed on to others, particularly nurses and physiotherapists; provision should be made for their training as well.

**Support groups.** An organization involving families and friends of individuals with CF, and other volunteers, should be established early. Such an organization can help ensure that CF services are developed by means of a partnership between professional and lay persons.

**Education.** Parents are the primary care providers of affected individuals and must be taught as much as possible about CF if they are to be as effective as possible. They should also be offered careful genetic counselling. Other lay persons having regular contact with the child, such as school teachers, need basic information so that they can appreciate the importance of regular medication, the need for a proper diet, and the possibility that the child may have recurrent bouts of illness.

**Evaluation.** It is important to build some form of regular evaluation and auditing into CF services from the beginning, so that their outcome can be monitored.

### **Contributing parties**

**Hospitals.** CF services are at least initially mainly hospital based, although in some situations participation by community health services can be very valuable. The hospital or hospitals providing the location for CF services need to be supportive.

**Medical schools.** If awareness of CF is to increase among medical professionals, it is important that medical schools play their part by including it in their curricula.

**CF associations.** A national organization with official status can be very helpful in raising public and professional awareness of the disease. As with support groups, a partnership should be maintained between lay organizations and professionals.

**CF physicians.** In addition to their involvement with a CF association of lay persons, clinicians dealing with the disease often find it helpful to hold meetings from time to time to share experiences and collaborate on further developments.

The role of other bodies, such as international organizations and pharmaceutical companies, is discussed below.

## **Implementation of cystic fibrosis services**

### **Improving awareness**

The primary responsibility for improving awareness about CF rests with the core staff of dedicated physicians, together with the national CF association. Initially it is wise to focus on obtaining the interest and support of other members of the medical profession, through local and national medical conferences. Leaders of medical schools and health services should be invited to these conferences, and perhaps also to regional and international CF meetings. Attempts should also be made to inform public health authorities and the general public about CF, but such attempts are more likely to succeed after a consensus has been reached within the medical profession. Public health authorities should subsequently be kept informed about national and international developments, particularly in treatment methods.

### **Estimating incidence**

On account of poor ascertainment and survival, the prevalence of CF in a community is no guide to its true incidence. It is essential for health authorities to know the scale of the problem if they are to make appropriate provisions for CF care. For this reason, in addition to ensuring that cases of CF are documented and notified to a central registry, consideration should be given to some form of population screening, preferably neonatal, as described above.

### **Increasing knowledge**

It is not enough simply to inform professionals and other groups that CF exists. They should also be given up-to-date information about its nature, diagnosis, management, severity, and prognosis. This can be achieved through presentations at meetings, conferences, and in national medical journals. Strategies for increasing the knowledge of key groups are described below.

**Professionals.** Medical conferences can be used not only to improve awareness about CF but also to develop knowledge about the disease among paediatricians and other physicians, as well others. Also, literature produced by the ICF(M)A, the International Physiotherapy Group for Cystic Fibrosis (IPG/CF), and the pharmaceutical industry may be useful. As mentioned above, it is important for CF to be included in medical school curricula, so that future doctors recognize the condition and know how to deal with it.

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**Lay persons.** It is important that parents and other volunteers have knowledge about CF that is as complete and up to date as possible. In this respect some of the more straightforward material designed for professionals is suitable also for educated lay persons. A manual for parents and families of CF patients is being prepared by WHO and the ICF(M)A, and other similar information can be obtained from pharmaceutical companies. Local CF groups also appreciate and derive great support from information talks arranged for them, for instance, by their national CF association.

**The public.** In the long-term, the most effective strategy for increasing the knowledge of the public probably is to ensure that human genetics is taught in schools, preferably using CF as an example of an autosomal recessive disorder. Newspapers, fund-raising events, radio, and television also provide opportunities for disseminating information to the general public.

### Reliable diagnosis

The importance of establishing a reliable diagnosis of CF using a properly conducted sweat test has been referred to above. In addition, diagnostic radiology

and laboratory facilities for sputum culture and pulmonary function tests are important for both initial diagnosis and diagnosis of complications. If available, a genetic reference laboratory capable of identifying CF mutations can be a valuable asset.

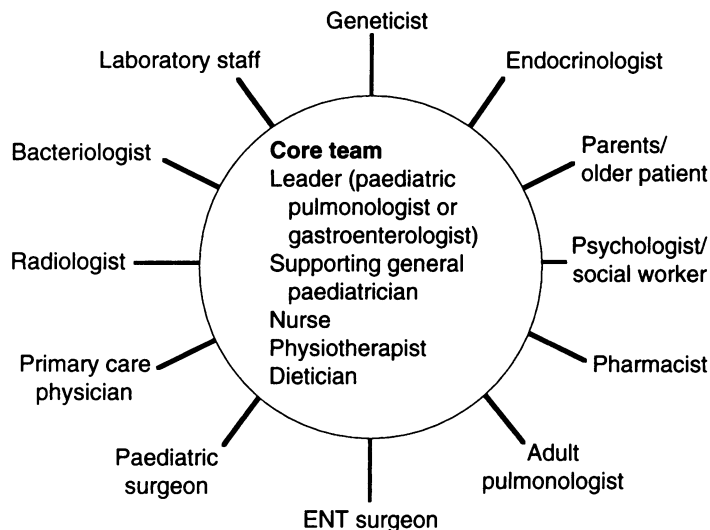
### Treatment centres

Centres for CF diagnosis and care should be developed. Diagnostic centres should be located in strategic locations in a country. Medical staff and those in allied professions should be trained to function as members of a team.

**CF team.** The CF team can be divided into core members, who have a continuing role, and fringe members, who contribute from time to time, as necessary. The core members (Fig. 1) must include:

- a pulmonary or gastrointestinal physician (usually a paediatrician) who functions as team leader;
- a general paediatrician, who may be senior or junior;
- a nurse (not necessarily specialized, but trained by the team);

Fig. 1. The cystic fibrosis treatment team (core and fringe members).



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- a physiotherapist to train and update patients and parents in the techniques of chest physiotherapy; and
- a dietician.

The fringe members include an adult physician (usually a pulmonologist), a bacteriologist, a geneticist, a psychologist and/or social worker, an endocrinologist, an ear, nose, and throat (ENT) surgeon, a paediatric surgeon, a pharmacist, a primary care physician, a radiologist, and laboratory staff.

The parents of an affected child, and older patients themselves, are also members of the team and their full involvement is essential to the success of therapy. The team should meet regularly and monitor its results, including patient and team performance.

**Facilities.** Therapeutic facilities (Fig. 2) must include the following:

- simple equipment for physiotherapy (physiotherapy should be adapted to the local popu-

lation; programmed exercise should be a major component);

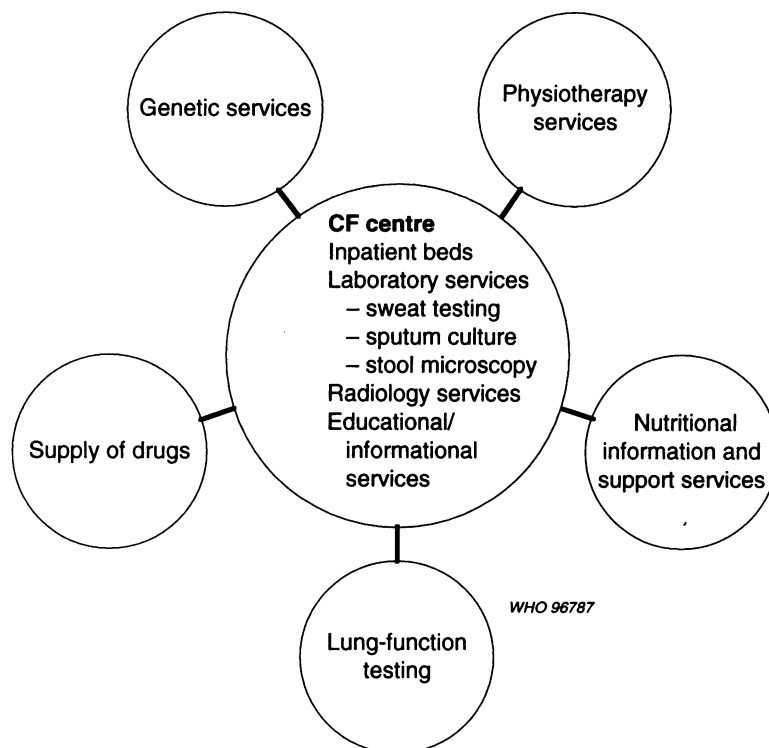
- nutritional information and support, emphasizing local resources;
- supplies of pancreatic enzymes and basic antibiotics, including anti-Pseudomonas agents;
- educational facilities for professionals and lay persons; and
- laboratory resources for sweat testing, bacteriology, and stool microscopy for fat.

The frequency with which a patient should be seen in the centre depends on his or her clinical condition, but on average should be every 6–12 weeks. In infants, the interval should not exceed 1 month.

### Registry

It is important to establish and maintain a CF registry. Essential data include name, date of birth, sex, age at diagnosis, and date of death. Other useful

Fig. 2. Therapeutic facilities for cystic fibrosis treatment.



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information includes the mode of presentation, the cause of death, and, when available, the genotype. Additional data can include information on height and weight at time of diagnosis and annual intervals, as well as bacteriological and lung function data.

### **Local research and evaluation**

Participation in simple research projects and the evaluation of performance engenders team spirit and helps morale. It is also of potential international importance in populations where the clinical profile of CF differs from that seen elsewhere, and may be correlated with differences in gene mutations.

### **Fund-raising**

However generous the support of health authorities, there is always a need for funds for publicity, family support, to promote new developments, and other activities. Raising funds should predominantly be the task of the national CF association, with occasional support from professionals.

## **Obstacles to the development of services**

Even where awareness of CF has been enhanced, the provision of CF services must take place in the context of the general health needs of a population, and there may still be substantial financial or other difficulties for the development of adequate services. Some of the problems that were identified at the meeting are described below.

**Public health priorities.** Public health priorities may be different from those of the families and professionals concerned with CF. This is more likely to occur when CF is perceived as a rare disease by public health authorities.

**Financial constraints.** There are invariably financial constraints in the budgets of existing medical services, and it is often difficult to add a completely new service.

**Dependence on developed countries.** There may be either a real or a perceived dependence on countries where CF services are more developed, and a corresponding delay in becoming self-sufficient. As more and more countries initiate their own CF services, it will be increasingly difficult for developed countries to give each of them even rudimentary support, and priorities for aid will doubtless take into account

both the potential for future services and the extent of existing local services.

**Health services.** Health services can be variable both between and within countries. Although it is generally accepted that individuals should not be penalized simply because they happen to be born or live in areas where CF services are less developed or non-existent, in practical terms there are significant differences in access to and quality of CF care.

**Diagnosis.** The diagnosis of CF is often missed or delayed in populations where there are high underlying prevalences of malnutrition, diarrhoea, and respiratory diseases. It may therefore take time before the clinicians dealing with these problems learn to identify the children who are atypical in presentation or resistant to conventional treatment.

**Lack of comparable models.** There is often a lack of comparable models for the management of chronic, debilitating diseases of childhood and adolescence (e.g. thalassaemia and haemophilia). The provision of a long-term supportive service for CF may therefore involve the acceptance of unfamiliar concepts.

**Population distribution.** The population distribution of CF can make access to diagnosis and treatment difficult for a large proportion of affected families.

**Supply of necessary drugs.** The supply of necessary drugs, particularly pancreatic enzymes, may be unreliable for a variety of reasons, including rigid adherence to essential drugs lists and a relatively small target population, reducing the profitability for importers. High-cost antibiotics should be avoided wherever possible.

### **Strategies for progress**

There are no easy solutions to many of the problems outlined above. Some, such as financial limitations, are common to developed and developing countries alike. In general, such problems can be overcome only by means of the strategies outlined under *Implementation of cystic fibrosis services* (see above).

## **Role of WHO, ICF(M)A, and other agencies**

### **WHO**

WHO should organize meetings and workshops on CF at the international level. With advice from the

ICF(M)A and others, it can identify experts able to advise countries and groups on the development of CF services. Through such activities, including the distribution and dissemination of information, national governments and health services can be made more aware of the existence of CF and the need for services.

### **ICF(M)A**

The ICF(M)A provides a forum for national CF organizations to share experiences and give mutual support. Through its Scientific/Medical Advisory Council, it disposes of a small research and development budget, which may be used to support visits of experts to developing countries or visits of young professionals to established centres in order to gain experience and training. In addition, it funds training courses for physiotherapists. ICF(M)A has produced CF literature suitable for dissemination to professional and lay people. Every 4 years, the ICF(M)A supports an international congress on CF which brings together interested professionals from all parts of the world.

### **IPG/CF**

With support from the ICF(M)A, the IPG/CF organizes occasional training courses for physiotherapists. These courses demonstrate the range of available techniques and help physiotherapists decide upon the most appropriate methods for their own populations. The IPG/CF holds annual meetings and has a worldwide membership. It has recently produced a small handbook which can be obtained upon request.<sup>a</sup>

### **Regional organizations**

Regional CF organizations exist in Europe and Latin America (European Working Group on Cystic Fibrosis, Latin American Working Group on Cystic Fibrosis). Each holds a regional/international meeting every year, except during years where there is an ICF(M)A international congress on CF. Attendance at these annual meetings is high, and presentations range from the latest developments in genetic research to new forms of treatment. Both regional organizations work closely with the ICF(M)A and can assist in identifying individuals and centres that are willing to take part in training programmes for professionals from developing countries.

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<sup>a</sup> For information, please contact J. Pryor, Physiotherapy Department, Royal Brompton Hospital, Sydney Street, London SW3 6NP, England.

### **The pharmaceutical industry**

The pharmaceutical industry can assist the development of CF services in several ways. First, various companies produce a wide range of scientific publications on CF, including interactive computer programs. The industry also helps track the incidence of CF through epidemiological studies, and may be willing to contribute towards the maintenance of a CF register. Although ethical constraints do not permit pharmaceutical companies to have access to the register, there is no objection to the industry's access to information about how many CF patients are in a particular country. Companies are also a traditional source of support for congresses, conferences, and local meetings, and occasionally will fund visiting speakers. They may also be willing to support short-term training of medical and allied personnel, and some produce information suitable for patients and families.

## **Conclusions and recommendations**

Knowledge of CF is poor in many of the countries where it is a significant burden on health. In these countries, many children with CF go undiagnosed and may die before their disease is recognized. Services for sweat testing, in particular, are poorly developed. Patients who are diagnosed are often treated inadequately or inappropriately. Life expectancy is consequently far below that which would be attainable with proper treatment. Support services for families affected by CF are usually nonexistent.

The following recommendations can be made.

- WHO and the ICF(M)A should continue to collaborate, and should focus their efforts on the needs of developing countries in particular.
- The resources of both organizations should be used to improve knowledge about CF, particularly in the medical and associated professions in developing countries. Both organizations should strive to draw the attention of the health authorities in those countries to the problem of CF.
- WHO and the ICF(M)A should use their resources to improve the training of professionals involved in the diagnosis and management of CF. The methods used could include training fellowships, visiting professorships, partnership and "twinning" arrangements between established and emerging CF centres, training courses, and assisted attendance of key personnel at international CF conferences. The

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organizations should continue to prepare and disseminate educational materials and guidelines produced either by themselves or in collaboration with others (such as the IPG/CF).

- At least one specialist CF clinic should be set up in each country whose population is affected by CF. Each centre should be led by a trained clinician, responsible for ensuring the provision of a reliable service for diagnosis and an adequate service for treatment. The CF centre should act as the focal point for setting up a national CF association, with lay and professional involvement.
- Regular contact should be established between CF centres within both countries and regions. Such contact is already well established in Western Europe and Latin America. A similar regional working group should be established in the Eastern Mediterranean Region.
- Consideration should be given to setting up limited neonatal screening projects in developing countries as a means of determining the incidence of CF and identifying affected infants.
- National and regional genetic laboratories should be encouraged to identify the specific CF mutations most commonly found in their populations, in order to help clinicians recognize the variations they may encounter.
- National registers of CF patients should be established in order to identify and predict the need for services and to monitor survival trends. WHO should support the distribution and dissemination of the data from countries providing such reports.
- National and international organizations should work in partnership with the pharmaceutical industry, which may support educational and epidemiological activities.

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## Acknowledgements

Dr I. Yacoub, Assistant Under-Secretary for Primary Care and Public Health, Ministry of Health, Manama, Bahrain, is thanked for kindly opening the meeting. The following are thanked for their expert advice and support throughout the meeting: Dr A.J. Al-Abassi, Chairman, Paediatric Department, Ministry of Health, Manama, Bahrain; Mr I. Akbari, Chief, International Health Relations, Ministry of Health, Manama, Bahrain; and Mr H. Dassel, Director, Segment Pancreas, Knoll AG, Ludwigshafen, Germany.

## Résumé

### Création de services de prise en charge de la mucoviscidose dans les pays en développement: Mémorandum d'une réunion conjointe OMS/AILM

Les précédentes réunions conjointes OMS/Association internationale de Lutte contre la Mucoviscidose (AILM) ont été consacrées à la distribution géographique de la mucoviscidose, à la lutte contre cette maladie, à son dépistage dans la population, aux méthodes actuelles de traitement et aux perspectives thérapeutiques. Bien que largement répandue, cette maladie présente des variations importantes d'incidence, les populations africaines et asiatiques étant relativement épargnées. En Europe de l'Ouest, aux Etats-Unis d'Amérique et en Australasie, la mucoviscidose est en général bien reconnue, et il existe des services appropriés de prise en charge. En revanche, il existe ailleurs dans le monde de vastes populations dans lesquelles la maladie est rarement reconnue, insuffisamment traitée et entraîne des décès et des souffrances évitables chez l'enfant comme chez l'adulte.

La présente réunion conjointe OMS/AILM avait pour mandat d'examiner ces problèmes et de déterminer des stratégies pour la création et le développement de services de prise en charge dans les pays en développement. Dans le présent contexte, "pays en développement" désigne un pays où la mucoviscidose est probablement beaucoup plus répandue que ne l'estiment le corps médical et le grand public, et où les services de prise en charge sont absents ou embryonnaires.

Il a été demandé aux participants de remplir des questionnaires sur la prise en charge de la mucoviscidose dans leur pays. Plusieurs problèmes fréquents ont été identifiés dans les pays en développement: sous-diagnostic; espérance de vie réduite chez les malades (par rapport aux pays "développés"); tableau clinique parfois différent de celui de la maladie "classique"; faible accès aux médicaments nécessaires; absence de services de prise en charge de la mucoviscidose ou de recherche fondamentale sur cette maladie. Dans un tel contexte, les objectifs des services de prise en charge de la mucoviscidose dans les pays en développement seront l'amélioration du diagnostic et de la survie, la définition du tableau clinique de la maladie dans la population locale, et la recherche de moyens d'améliorer l'approvisionnement en médicaments.

Pour atteindre ces objectifs, il faut disposer d'outils simples permettant d'identifier les personnes touchées par la maladie. Le principal test de

diagnostic est le test à la sueur, qui consiste à recueillir la sueur dans des conditions normalisées et à analyser sa teneur en chlorure de sodium. Le dépistage néonatal constitue une autre possibilité de diagnostic et peut être particulièrement intéressant pour donner une estimation de la charge que représente la mucoviscidose dans une population où cette maladie est sous-diagnostiquée. Il existe deux méthodes: mesure de la trypsine immunoréactive dans des prélèvements de sang sur papier filtre, et mesure de l'albumine dans le méconium au moyen de bandelettes réactives (méthode moins onéreuse mais dont le taux de faux-négatifs est d'environ 15%). Néanmoins, dans certaines populations où l'on estime que 90% des malades décèdent sans que la maladie ait été diagnostiquée (par exemple en Amérique latine), le test sur le méconium permet d'améliorer le dépistage à un coût relativement faible.

Il est en outre nécessaire d'établir des centres spécialisés dans le diagnostic et le traitement de la mucoviscidose. Les centres de diagnostic devront être situés dans des endroits soigneusement sélectionnés. Un ou deux médecins motivés peuvent constituer le point de départ d'un service de diagnostic et de traitement. Dès le démarrage de tels services, il faudra créer une organisation nationale de lutte contre la mucoviscidose rassemblant des familles et des proches de malades et d'autres bénévoles, de façon à établir un partenariat entre les professionnels de la santé et le public.

Le personnel médical et paramédical devra être formé à travailler en équipe, celle-ci étant composée de membres permanents et de membres occasionnels. Parmi les membres permanents figureront un médecin spécialisé en pneumologie et/ou gastro-entérologie (en général un pédiatre) qui dirigera l'équipe; un pédiatre généraliste; un infirmier (non nécessairement spécialisé, mais qui sera formé par les membres de l'équipe); un physiothérapeute pour enseigner ou rappeler aux malades et à leur famille les techniques de physiothérapie thoracique, et un diététicien.

Les membres occasionnels seront un médecin (habituellement un pneumologue) spécialiste de l'adulte, un bactériologiste, un généticien, un psychologue et/ou un travailleur social, un endocrinologue, un chirurgien en oto-rhino-laryngologie, un chirurgien en pédiatrie, un pharmacien, un médecin de soins de santé primaires, un radiologue, et des personnels de laboratoire. Les parents des enfants malades, et les malades plus âgés eux-mêmes, feront également partie de l'équipe et leur participation sera indispensable à la réussite du traitement. L'équipe devra se

réunir régulièrement pour faire le point sur ses résultats, au niveau du malade comme de l'équipe elle-même.

Les ressources thérapeutiques dont disposera l'équipe devront comprendre: du matériel simple pour la physiothérapie; une information et un soutien nutritionnels, en insistant sur les ressources locales; un approvisionnement en enzymes pancréatiques et en antibiotiques de base, y compris des médicaments anti-*Pseudomonas*; du matériel éducatif à l'intention des professionnels et du public; des moyens de laboratoire pour le test à la sueur, les analyses bactériologiques et la recherche des graisses dans les selles.

Il est important d'établir et de tenir à jour un registre de la mucoviscidose; on notera pour chaque malade le nom, la date de naissance, le sexe, l'âge au diagnostic, et la date du décès. On pourra aussi indiquer le tableau clinique de la maladie, la cause du décès, le génotype si possible, et ajouter des informations sur la taille et le poids au moment du diagnostic et à intervalles annuels, ainsi que des données bactériologiques et des données sur la fonction pulmonaire.

La sensibilisation du corps médical et du public au problème de la mucoviscidose incombe au personnel permanent constitué de médecins motivés, avec le soutien de l'association nationale de lutte contre la mucoviscidose. On cherchera dans un premier temps à susciter l'intérêt et le soutien des autres membres du corps médical en organisant des conférences au niveau local et national. On cherchera également à informer les autorités de santé publique et le grand public lui-même, mais cette tâche sera plus aisée lorsqu'il existera un consensus au niveau du corps médical. Les autorités de santé publique devront par la suite être tenues informées de l'état des connaissances sur la maladie, notamment en ce qui concerne les méthodes de traitement.

Les documents publiés par l'AILM, par l'International Physiotherapy Group for Cystic Fibrosis, et par l'industrie pharmaceutique peuvent être utiles. Certains documents destinés aux professionnels de la santé peuvent également convenir au grand public cultivé. L'AILM prépare actuellement un manuel destiné aux parents et aux familles des malades, et les laboratoires pharmaceutiques peuvent fournir des brochures du même type.

Les groupes locaux de lutte contre la mucoviscidose font volontiers appel aux débats à but informatif. Les journaux, les manifestations spéciales organisées en vue de la collecte de fonds, la radio et la télévision sont autant de moyens de diffuser l'information.

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Il ne suffit pas d'informer les professionnels et le public de l'existence de la mucoviscidose. Il faut fournir une information à jour sur la nature, le diagnostic, la prise en charge, la gravité et le pronostic de la maladie. On peut aussi organiser des conférences destinées aux pédiatres et autres médecins. Il importe également de faire figurer cette maladie au programme des études médicales.

Les recommandations suivantes ont été formulées lors de la réunion:

- L'OMS et l'AILM devront poursuivre leur collaboration et axer leurs efforts sur les besoins des pays en développement.
- Ces deux organisations devront utiliser leurs ressources pour améliorer l'état des connaissances sur la mucoviscidose, en particulier chez les membres des professions médicales et paramédicales des pays en développement. Elles s'emploieront à attirer l'attention des autorités sanitaires de ces pays sur le problème de la mucoviscidose.
- L'OMS et l'AILM devront utiliser leurs ressources pour améliorer la formation des professionnels chargés du diagnostic et de la prise en charge de la mucoviscidose. Elles feront appel à des bourses d'études, des échanges de professeurs, des accords de partenariat et de jumelage entre les centres existants et les centres nouvellement créés, des cours de formation et une participation des personnels clés aux conférences internationales. L'OMS et l'AILM devront continuer à préparer et diffuser des matériels éducatifs et des directives qu'elles prépareront sous leur propre nom ou en collaboration avec d'autres organismes.
- Un dispensaire spécialisé au moins devra être établi dans tous les pays dont la population est touchée par la mucoviscidose. Chaque centre sera dirigé par un médecin qualifié, qui sera chargé d'organiser un service fiable de diagnostic et un service adéquat de traitement. Le centre de prise en charge de la mucoviscidose devra servir de point focal pour la création d'une association nationale de lutte contre la mucoviscidose, à laquelle participeront des professionnels de la santé et des membres du grand public.

- Des contacts réguliers devront être établis entre les centres d'un même pays et d'une même région. De tels contacts sont déjà bien établis en Europe de l'Ouest et en Amérique latine; un groupe de travail régional du même type devra être établi dans la Région de la Méditerranée orientale.

- On envisagera d'établir des projets limités de dépistage néonatal dans les pays en développement en tant que moyen de déterminer l'incidence de la mucoviscidose et d'identifier les nourrissons atteints.

- Les laboratoires nationaux et régionaux de génétique devront être encouragés à rechercher les mutations géniques à l'origine de la mucoviscidose les plus couramment rencontrées dans la population, afin d'aider les médecins à reconnaître les divers aspects cliniques que peut présenter la maladie.

- Des registres nationaux de la mucoviscidose devront être établis afin d'identifier et de prévoir les besoins en services et de suivre les tendances de la survie. L'OMS devra encourager la collecte et la diffusion des données en provenance des pays fournissant de tels rapports.

- Les organisations nationales et internationales devront œuvrer en partenariat avec l'industrie pharmaceutique, qui pourra elle-même soutenir des activités éducatives et épidémiologiques.

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## References

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