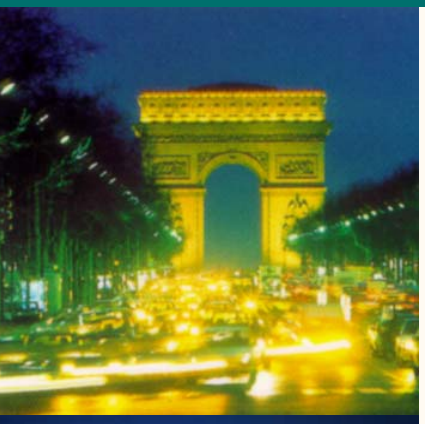
DRUG INFORMATION ASSOCIATION

Dedicated to Your Information & Advancement

www.diahome.org

May 10-11, 2004 - Hotel Sofitel Paris Forum Rive Gauche, Paris, France



Assessing Treatment Impact Using PROs: Challenges in Study Design, Conduct and Analysis

This DIA workshop is being organised in cooperation with the ERIQA Group

Programme Committee

Eric Abadie, MD, MBA, French Medicines Agency (AFSSAPS), France
Lynda Bryant-Comstock, MPH, MS, GlaxoSmithKline Inc., USA
Olivier Chassany, MD, PhD, Saint-Louis University Hospital Paris, France
Asha Hareendran, PhD, Pfizer Global Pharmaceuticals, UK
Bernard Jambon, MAPI Group, France

Advisory Committee

Catherine Acquadro, MD, MAPI Research Institute, France
Giovanni Apolone, MD, Mario Negri Institute, Italy
Dominique Dubois, MD, FFPM, MBCPM, Johnson & Johnson Pharmaceutical Services LLC, Belgium
Annoesjka Novak, MSc, NV Organon, The Netherlands

Background

In the past 20 years, the growth of patient-reported outcome (PRO) instruments being used in multinational clinical trials has increased dramatically. As a result, regulatory authorities are increasingly faced with having PROs as an additional outcome measure for the evaluation of new therapies. Moreover, PROs are being recognized as an important evaluation criteria. However, there are key issues that need to be addressed between manufacturers, health care providers and regulatory authorities.

Overview

This workshop will provide an overview of current issues in clinical trial research using patient-reported outcomes (PROs). Examples of industry use of patient-reported outcomes to support European and FDA medical product approval decisions will be presented.

Conference Objectives

- To discuss CPMP, national regulatory authorities, and FDA's experience in PRO data review to support medical product approval
- To explain challenges in PRO study design, conduct analysis and interpretation when assessing treatment impact
- To recognize when PROs may add value to the evaluation of treatment effectiveness and thereby strengthen an application for registration
- To generate discussions between regulators, academics and members of pharmaceutical industry

Who Should Attend?

This conference should be attended by professionals such as clinical trial investigators and reviewers, outcomes researchers, medical product industry professionals and by professionals from related areas.

Table Top Exhibition

The DIA will provide the opportunity for pharmaceutical support organisations to exhibit their materials and services at this meeting. To obtain details on exhibiting space and facilities, interested exhibitors should contact:
Drug Information Association, European Branch Office - Cornelia Naumann
Elisabethenanlage 11, Postfach, 4002 Basel, Switzerland
Tel.: +41 61 225 51 61 - Fax: +41 61 225 51 45 - E-mail: cornelia.naumann@diaeurope.org

ABOUT THE DRUG INFORMATION ASSOCIATION

With more than 27,000 members worldwide, the Drug Information Association (DIA) is the premier member-driven organization encompassing the full continuum of disciplines in the pharmaceutical and related industries. The mission of DIA is to serve and develop members by providing a neutral, global forum that promotes the exchange of information critical to their professional performance and achievement. The goal of DIA is to be the most effective means for members to obtain the knowledge they need to advance their career, their profession, and their organization.

VISIT OUR WEBSITE WWW.DIAHOME.ORG

08:00 **TUTORIAL REGISTRATION & WELCOME COFFEE**

08:30 **TUTORIAL**

EDUCATIONAL PROGRAMME ON PATIENT REPORTED OUTCOMES IN CLINICAL TRIALS

Tutorial Instructors

Catherine Acquadro, MD, Scientific Advisor, MAPI Research Institute, France

Olivier Chassany, MD, PhD, Associate Professor in Therapeutics, Director, Clinical Research Dept, Direction de la Politique Médicale de l'Assistance Publique - Hôpitaux de Paris, France

(15 min. coffee break at approx. 10:30)

NOTE - LIMITED TO 15 ATTENDEES! FIRST COME, FIRST SERVED.

A recent survey of European regulators conducted by the ERIQA (European Regulatory Issues on Quality of Life Assessment) Group showed that health-related quality of life (HRQL) training was a key issue for regulators involved in the review of drug approval files.
To meet the need of clinical trials reviewers, ERIQA and the Cochrane Health-Related Quality of Life Methods Group designed an educational programme on PRO based on the Workmats and developed with the feedback from the health authorities, including the FDA and European Agencies.

Workmats have been designed for **interactive learning within a group** in order to facilitate the understanding of key concepts. This group-learning methodology stimulates small-group discussions and interaction, and enables better understanding and thought processes. Each Workmat is a large sheet of paper divided into three parts: 1. a brief background introducing the theme; 2. a summary of objectives to achieve during the discussion and, 3. a list of assignments to be completed by the participants through group discussions which will enable them to arrive at a joint conclusion (i.e. the key learning points).

Objectives are to help pharmaceutical companies, reviewers and investigators of clinical trials acquire the skills needed to assess PRO included in regulatory files and publications, to facilitate decisions made by health authorities and health-care providers, and to improve the dialogue between regulators, members of pharmaceutical companies, and health-care providers.

Upon completion of this educational programme, participants will be familiar with the key issues required to evaluate PRO data from clinical trials, and should be able to:

- Recognize the types of PRO's used in clinical trials;
- Use tools to conduct a systematic review of PRO-based data from a clinical trial, including resources to help identify existing PRO's and key references in PRO development, validation, application and interpretation.

Who should attend?

This introductory course is specifically designed for regulators, academics and manufacturers who have novice to intermediate experience in PRO evaluation. This course will be beneficial to manufacturers who are in clinical research, project management, regulatory affairs and marketing.

12:30 **LUNCH FOR TUTORIAL PARTICIPANTS**

12:30 WORKSHOP REGISTRATION

14:00 DIA OPENING
Wayne Nitchuk, DIA European Branch Office, Switzerland

14:15 DIA SIAC IMPaCT OPENING
Bernard Jambon, MAPI Group, France

14:25 MEETING OVERVIEW AND OBJECTIVES
Catherine Acquadro, MAPI Research Institute, France

14:30 **Session 1**

PATIENT REPORTED OUTCOMES: WHEN DO THEY ADD VALUE?

Session Chairperson:
Cristina Sampaio, Instituto Nacional da Farmacia e do Medicamento, Portugal
Determinants of the added value of a multidimensional health-related quality of life and other PRO assessment will be identified and presented using examples from oncology and respiratory diseases.

Overview: Examples of When PROs Add Value
Asha Hareendran, Pfizer Global Pharmaceuticals, UK

Examples in Respiratory Diseases
Thys van der Molen, University of Groningen, Department of General Practice, The Netherlands

Examples in Oncology
Pierre-Philippe Sagnier, Bayer Health Care, UK

16:00 **COFFEE BREAK**

16:30 **Session 2**

HOW TO INTEGRATE PATIENT REPORTED OUTCOMES IN INTERNATIONAL TRIALS? REGULATORY ISSUES

Importance of a Guidance in PROs: PROS and CONS
Dominique Dubois, Johnson & Johnson Pharmaceutical Services LLC, Belgium

Practical Experience from a Reviewer: Reviewers - What Do They Expect?
Olivier Chassany, Saint-Louis University Hospital Paris, France and Giovanni Apolone, Mario Negri Institute, Italy

18:00-19:00 **RECEPTION**

Please note that this programme is being updated on a regular basis on the DIA Home Page www.diahome.org

Tuesday, May 11, 2004

08:30 WELCOME COFFEE

09:00 Session 3

HOW TO INTEGRATE PATIENT REPORTED OUTCOMES IN INTERNATIONAL TRIALS? METHODOLOGICAL ISSUES

Session Chairperson:

Peter Fayers, University of Aberdeen, Medical School, UK

PRO assessments in clinical trials raise a number of challenging methodological issues. This session explores topics in the design, analysis and interpretation of studies that use PRO endpoints.

Clinical Trial Design and Cross-cultural Issues for PROs
Linda Abetz, MAPI Values Ltd., UK

Statistical Issues: Missing Data, Multiplicity
Peter Fayers, University of Aberdeen, Medical School, UK

Interpretation: Clinical Significance: What Does It Mean?
Patrick Marquis, MAPI Values, US

11:00 COFFEE BREAK

11:30 Session 4

USE OF PATIENT REPORTED OUTCOMES TO SUPPORT EUROPEAN AND FDA APPROVAL DECISIONS

Session Chairperson:

Eric Abadie, French Medicines Agency (AFSSAPS), France

Representatives from FDA and Regulatory Authorities will address the major issues associated with using PROs as endpoints in studies to support medical product approval. In addition a clinician involved in clinical trials and using PROs in his practice will present his views.

QoL from a Regulatory Perspective
Andre J.A. Elferink, Medicines Evaluation Board, The Netherlands

The Role of Patient Reported Outcomes in US Drug Approval and Labeling Decisions
Laurie B. Burke, FDA, USA

13:00 Panel Discussion

CONCLUDING REMARKS

Panelists:
Neil Aaronson, The Netherlands Cancer Institute, The Netherlands
Olivier Chassagny, Saint-Louis University Hospital Paris, France
Ingela Wiklund, AstraZeneca, Sweden

13:30 LUNCH BREAK

14:30 - 16:00 PARALLEL SESSIONS

Participants can only attend one Parallel Session and should indicate their choice on the registration form

PARALLEL SESSION 1

GASTROINTESTINAL DISORDERS

Session Chairperson:

Olivier Chassany, Saint-Louis University Hospital Paris, France

To explain the added value of PROs compared to other outcomes in the context of gastrointestinal disorders; to define the disorders in which the PROs may have the greatest interest in the assessment of the value of a drug; and to present practical examples where PROs have been successful and helpful.

The Patient's Perspective in Gastrointestinal Disorders: From Top to Bottom
Olivier Chassany, Saint-Louis University Hospital Paris, France

Added-value of PROs in Gastrointestinal Disorders: Perspective from a Pharmaceutical Representative
Ingela Wiklund, AstraZeneca, Sweden

Added-value of PROs in Gastrointestinal Disorders: Perspective from a Clinician
Karsten Lauritsen, Odense University Hospital, Denmark

PARALLEL SESSION 2

RESPIRATORY DISEASES

Session Chairperson:

David Lyons, Irish Medicines Board, Ireland

Although patient reported outcomes have traditionally been recorded in clinical trials of treatments for respiratory diseases they have, arguably, been second class citizens in the evaluation of efficacy. This session will examine whether there are underlying reasons for the apparent neglect of PROs, will define what PROs are currently used in regulatory and scientific practice and what they add to traditional evaluations such as pulmonary function and radiology. Speakers will examine the issues from a regulatory, clinical, and industry point of view with emphasis on asthma and COPD. Audience participation and dialogue is encouraged.

QoL in Chronic Bronchial Diseases
Pascal Chanez, Hospital Arnaud de Villeneuve, France

PROs in Respiratory Medicine: A Neglected Friend?
David Lyons, Irish Medicines Board, Ireland

Experience of PROs in Respiratory Disease - What Can We Learn ?
Michael Spencer, GlaxoSmithKline, UK

PARALLEL SESSION 3

ONCOLOGY

Session Chairperson:

Neil Aaronson, The Netherlands Cancer Institute, The Netherlands

This session will include three presentations addressing: (1) the current state-of-the art of health-related quality of life (HRQL) assessment in oncology; (2) the added-value of HRQL assessments in clinical trials in oncology, with an emphasis on advanced breast cancer; and (3) the value of HRQL assessments from the point of view of a clinical researcher and practitioner. In addition to the formal presentations (each of approximately 20 minutes), there will be ample time (about 30 minutes) for group discussion.

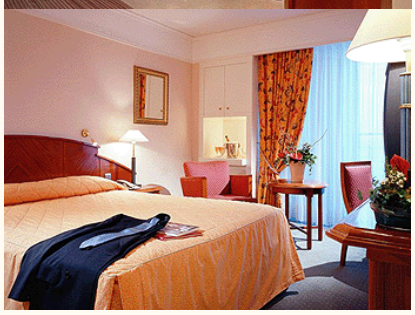
Health-related Quality of Life (HRQL) Assessment in Clinical Oncology Research and Practice: Current Status and Future Challenges
Neil Aaronson, The Netherlands Cancer Institute, The Netherlands

Do PROs Have Incremental Value in Oncology? The Case of Quality of Life Measures in Breast Cancer
Giovanni Apolone, Mario Negri Institute, Italy

A Clinician's View of HRQL in Oncology: Is it Worth it? Does it Matter?
Peter G. Harper, Guys and St. Thomas's Hospitals, UK

16:00 END OF WORKSHOP

Hotel & Travel Information



The DIA has blocked a limited number of rooms at the:

Hotel Sofitel Paris Forum Rive Gauche
Reservation Department,
17, boulevard Saint-Jacques
75014 PARIS, France

at the special rate of:
Single or Double Room: EUR 180.00
(inclusive VAT, Buffet Breakfast at EUR 20.00 is not included)

Attendees must make their own hotel reservation
by telephone: +33 1 40 78 78 40, or by telefax: +33 1 40 78 78 04
referring to the DIA Workshop on
"**Assessing Treatment Impact Using PROs: Challenges in Study Design, Conduct and Analysis**".

A deposit payment for one night must be made to secure the reservation by providing, with signature, the name, number and expiry date of your credit card.

IMPORTANT: To be assured of accommodation in the Hotel Sofitel Paris Forum Rive Gauche, the registrants are recommended to complete their reservation, if possible, by April 9, 2004.

The Hotel Sofitel Paris Forum Rive Gauche is situated in a residential area near Montparnasse and the Quartier Latin, near the underground station Saint-Jacques and Glacière. There are also direct links with Roissy-Charles de Gaulle Airport (45 minutes by RER) and Orly Airport (30 minutes by RER) and 30 minutes by Orlybus stopping in front of the hotel.

By car: Access to Paris from boulevard peripherique. From the North take motorway, following Paris Est signs to the porte d'Italie. Follow signs to place d'Iatlie, then take boulevard Blanqui towards Denfert-Rochereau. From the East via motorway A4 : at porte de Bercy, follow peripherique sud sign to porte d'Italie. Follow signs to place d'Italie, then take boulevard Blanqui towards Denfert-Rochereau. From the South via motorway A6 access through porte d'Orleans.

Workshop Cancellation Policy

ON OR BEFORE MAY 5, 2004
An administrative fee will be deducted from the registration fee:
Member/Nonmember = EUR 200.00
Government/Academia/Nonprofit (Member/Nonmember) = EUR 100.00
Tutorial only = EUR 50.00
Registrants who do not cancel by the date above and do not attend, will be responsible for the full registration fee. Registrants are responsible for cancelling their own hotel reservations. Cancellations must be in writing. You may transfer your registration to a colleague at any time. Please notify DIA of any such substitutions as soon as possible. DIA reserves the right to alter the venue, if necessary. If an event is cancelled, DIA is not responsible for airfare, hotel or other costs incurred by registrants.

Registration Form - I.D. Code # 04105

Fax to: +41 61 225 51 52

DIA Drug Information Association

Assessing Treatment Impact Using PROs: Challenges in Study Design, Conduct and Analysis

May 10-11, 2004 - Hotel Sofitel Paris Forum Rive Gauche, Paris, France

Registration will be accepted by mail, fax or email

Registration Fees

If DIA cannot verify your membership upon receipt of registration form, you will be charged the nonmember fee. Registration includes workshop material, coffee-breaks, luncheon and reception.

Member	Fee: EUR 1'250.00	19.6% VAT: EUR 245.00	Total Amount to be paid: EUR 1'495.00 ❑
Nonmember	Fee: EUR 1'625.00	19.6% VAT: EUR 318.50	Total Amount to be paid: EUR 1'943.50 ❑
Charitable Nonprofit/Academia (Full-Time), Member	Fee: EUR 937.50	19.6% VAT: EUR 183.75	Total Amount to be paid: EUR 1'121.25 ❑
Charitable Nonprofit/Academia (Full-Time), Nonmember	Fee: EUR 1'218.75	19.6% VAT: EUR 238.88	Total Amount to be paid: EUR 1'457.63 ❑
Government Member	Fee: EUR 625.00	19.6% VAT: EUR 122.50	Total Amount to be paid: EUR 747.50 ❑
Government Nonmember	Fee: EUR 812.50	19.6% VAT: EUR 159.25	Total Amount to be paid: EUR 971.75 ❑
I will attend the Tutorial on May 10, 2004 *(Registration includes luncheon)*	Fee: EUR 175.00	19.6% VAT: EUR 34.30	Total Amount to be paid: EUR 209.30 ❑

Please indicate which Concurrent Session you wish to attend: Parallel Session 1 ❑ Parallel Session 2 ❑ Parallel Session 3 ❑

Join DIA now to qualify for the member fee, save and receive all the benefits of membership for a full year! www.diahome.org/docs/Membership **EUR 130.00** ❑

Please tick the applicable category:
❑ Academia ❑ Government ❑ Industry ❑ CSO ❑ Student

Total Remittance EUR _____

Registrant

❑ Prof. ❑ Dr. ❑ Ms. ❑ Mr.

Last Name		Company	
First Name & Middle Initial		Job Title	
Street Address / P.O. Box		Postal Code	
City		Country	
(*)Telephone		(*)Telefax	
E-Mail			

(*) A telephone and fax number are required for faxed confirmation

Payment

❑ **Cheques** should be made payable to: Drug Information Association. Mail your cheque together with the registration form to facilitate identification of attendee to: DIA, Postfach, 4002 Basel, Switzerland.

❑ **Bank transfers should be made in EURO to following bank:**
UBS, Postfach, 4002 Basel, Switzerland - DIA Account Number: 233-635384.60C - SWIFT: UBSWCHZH80A - IBAN: CH96 0023 3233 6353 8460 C
Your name and company must be included on the transfer document to ensure payment to your account.

❑ **Please charge my credit card -** Credit card payments by VISA, Mastercard or AMEX can be made by completing the relevant details below. Please note that other types of credit card cannot be accepted.

❑ Visa ❑ MC ❑ AMEX

Card Number ☐☐☐☐☐☐☐☐☐☐☐☐☐☐☐☐ Exp.date ☐☐/☐☐

Cardholder's Name

Please indicate other method of payment: ❑ Cheque ❑ Bank transfer

Date: Cardholder's Signature:

VAT N° FR 30 447 740 366

Group Discount Available! Send 3, the 4th is FREE!

Register 3 individuals from the same company and receive complimentary registration for a 4th! **All 4 individuals must be registered and prepay at the same time.**
DIA will apply the value of the lowest applicable fee to this complimentary registration; It does NOT include fees for optional events or DIA membership. Substitutions of enrolled delegates of similar membership may be made at any time. **Group registration is not available online.** To take advantage of this offer, please make a copy of this registration form for EACH of the four registrants from your company and return them together to DIA.

❑ **Please indicate that this form is part of a group registration by ticking this box.**

Please indicate the full names of the other three registrants from your company (must represent the same company as registrant #1)

2. Name 3. Name 4. Name

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UPCOMING DIA EUROPEAN EVENTS

MARCH - NOVEMBER 2004

This calendar contains a listing of the DIA events currently scheduled. Complete programmes are generally available three months prior to the event. If you are interested in receiving programme information for an event, please do not hesitate to contact your nearest DIA office. More workshops and educational seminars being planned, watch for the announcements. All programmes are posted on our Web Page www.diahome.org which is regularly updated.

MARCH 10-12, 2004
16TH ANNUAL EUROMEETING PRAGUE 2004
EXPANDING HORIZONS - HOPES AND CHALLENGES
CONGRESS CENTRE, PRAGUE, CZECH REPUBLIC

MARCH 15-16, 2004
APPLIED EPIDEMIOLOGY TRAINING COURSE
HOTEL CROWNE PLAZA, AMSTERDAM, THE NETHERLANDS

APRIL 19-21, 2004
15TH INTERNATIONAL WORKSHOP ON STATISTICAL METHODOLOGY IN CLINICAL R&D
THE BURLINGTON HOTEL, DUBLIN, IRELAND

APRIL 21, 2004
A HALF DAY SURVIVAL COURSE IN STATISTICS FOR GENOMIC DATA
THE BURLINGTON HOTEL, DUBLIN, IRELAND

APRIL 22-23, 2004
4TH INTERNATIONAL WORKSHOP ON STATISTICAL METHODOLOGY IN NON-CLINICAL R&D
THE BURLINGTON HOTEL, DUBLIN, IRELAND

APRIL 26-29, 2004
SPECIAL TRAINING COURSE ON
US REGULATORY AFFAIRS
PHASE I - IND PHASE
PHASE II - NDA PHASE INCLUDING CTD
HEIDELBERG MARRIOTT HOTEL, HEIDELBERG, GERMANY

MAY 10-11,2004
ASSESSING TREATMENT IMPACT USING PRO'S: CHALLENGES IN STUDY DESIGN, CONDUCT AND ANALYSIS
HOTEL SOFITEL PARIS FORUM RIVE GAUCHE, PARIS, FRANCE

JUNE 7, 2004
TRAINING COURSE ON EUROPEAN REGULATORY AFFAIRS
SCANDIC HOTEL COPENHAGEN, COPENHAGEN, DENMARK

SEPTEMBER 28-30, 2004
MIDDLE EAST REGULATORY CONFERENCE MERC 6
THE JUMEIRAH CONFERENCE CENTER, DUBAI, U.A.E.

OCTOBER 6-8, 2004
TRAINING COURSE ON PRACTICAL GCP COMPLIANCE AUDITING OF TRIALS AND SYSTEMS
HOTEL COPTHORNE TARA, LONDON, UK

OCTOBER 14-15, 2004
11TH SEMINAR ON MEDICAL APPROACH IN DIAGNOSIS AND MANAGEMENT OF ADRS
HOTEL SOFITEL PARIS FORUM RIVE GAUCHE, PARIS, FRANCE

NOVEMBER 8, 2004
THREE WORLDS ONE VOICE - FIRST JOINT ANNUAL CONFERENCE
- **CLINICAL DATA MANAGEMENT**
- **INFORMATION TECHNOLOGY**
- **VALIDATION**

RAI AMSTERDAM INTERNATIONAL EXHIBITION & CONGRESS CENTER, AMSTERDAM, THE NETHERLANDS

NOVEMBER 29, 2004
TRAINING COURSE ON EUROPEAN REGULATORY AFFAIRS
HOTEL RENAISSANCE PARIS LA DÉFENSE, PARIS, FRANCE

NOVEMBER 29-30, 2004
FIRST DIA MULTI-TRACK MEETING ON CLINICAL TRIALS AND PHARMACOVIGILANCE
HOTEL SOFITEL PARIS FORUM RIVE GAUCHE, PARIS, FRANCE

PLEASE NOTE THAT THESE PROGRAMMES ARE BEING UPDATED ON A REGULAR BASIS ON OUR WEBSITE

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