

# X Jornada d'actualització sobre l'hemofília



**“Consell Consultiu de la Fundació  
Catalana de l’Hemofília. Finalitat i línies  
d’actuació prioritàries”**

**Dr. Rafael Parra**

Hematòleg

Cap de Centre. BST-Vall d’Hebron

President del Consell Consultiu de la FPCH

# OBJECTIUS I FUNCIONS DEL CONSELL CONSULTIU DE LA FUNDACIÓ PRIVADA CATALANA DE L'HEMOFÍLIA

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**Respondre a les qüestions científiques** plantejades pel Patronat de la FPCH

Elaborar **documents de consens, dictàmens, informes i declaracions científiques** derivades de les consultes que sorgeixen des de la FPCH

Organitzar i **dissenyar el programa** de la jornada anual i d'altres esdeveniments de caràcter similar

Proposar al Patronat de la FPCH **noves línies d'actuació** per la **promoció de la salut** de les persones amb coagulopaties Congènites.

# COMPOSICIÓ DEL CONSELL CONSULTIU

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El Consell Consultiu està format per professionals amb reconeguda trajectòria científica en el diagnòstic i tractament de persones amb coagulopaties congènites i/o vinculats a l'assistència d'aquest col·lectiu

President: Dr. Rafael Parra  
Secretari: Dr. Jose Mateo  
Vocals: Dra. Carme Altisent  
Dr. Ruben Berrueco  
Dr. Victor Jimenez-Yuste  
Dr. Miquel Rutllant  
Dra. Amparo Santamaria

## PROPOSTES DE NOUS PROJECTES

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**1. CREACIÓ DEL REGISTRE DE PACIENTS AMB  
COAGULOPATIES CONGÈNITES DE CATALUNYA**

**2. GRUP DE TREBALL PER LA REHABILITACIÓ EN  
L'ARTROPATIA HEMOFÍLICA**

# 1. CREACIÓ DEL REGISTRE DE PACIENTS AMB COAGULOPATIES CONGÈNITES DE CATALUNYA



## REGISTRE DE PACIENTS: DEFINICIÓ

Un registre és una **base de dades** de individus diagnosticats amb hemofília o d'altres coagulopaties congènites.

**Recull informació estandarditzada** sobre detalls personals: edat, sexe, tipus de malaltia, gravetat, tractament, complicacions (inhibidors, artropatia, VIH, hepatopatia...)

**Consentiment informat.**

**Les dades són anònimes.**

# World Bleeding Disorder Registry (WBDR) | Section 1



## SECTION 1.1: DEMOGRAPHY & DIAGNOSTIC INFORMATION



Centre ID:  Patient ID:

### Baseline Characteristics

Date of baseline data collection:     
Month (MM) Day (DD) Year (YYYY)      Date of birth:     
Month (MM) Day (DD) Year (YYYY)

Sex:  Hemophilia type:  Severity:

Measured clotting factor activity  Result: % FVIII / IX:C  %      Measured Activity Date:     
Month (MM) Day (DD) Year (YYYY)

FVIII Antigen  Result: % FVIII:Ag  %      Date:     
Month (MM) Day (DD) Year (YYYY)

Twin with same deficiency:  Blood group:

HIV status:  HCV antibody:  HCV PCR:

Mutation known:  Intron 22 inversion:

Mutation comment

Weight (3 digits, 1 decimal):  kg      Height (3 digits, 1 decimal):  cm

## SECTION 1.4: ANNUAL FOLLOW UP DATA

Centre ID:  Patient ID:

### Prophylaxis Factor Replacement Therapy

Please record data on Prophylaxis Replacement Therapy used during the reporting period. Do not include data that was entered in the First 50 Exposures table.

None       Unknown

Type of Prophylaxis	Start date of therapy	End date of therapy	# Exposures*	Product type	Brand name	Dose of prophylaxis, IU/kg or ml	Frequency
—select—	<input type="checkbox"/> Ongoing from previous visit	<input type="checkbox"/> Ongoing		—select—			—select—
—select—	<input type="checkbox"/> Ongoing from previous visit	<input type="checkbox"/> Ongoing		—select—			—select—

\* An exposure is defined as a 24-hour period in which FVIII / IX containing product is given to a patient. (Blanchette, VS et al. 2014)

### Other Interventions Requiring Factor Replacement Therapy

Please record any **other** intervention that the patient underwent requiring Factor Replacement Therapy during the reporting period. Do not include data that was entered in the First 50 Exposures, Episodic or Prophylaxis tables.

None       Unknown

Procedure	Start date of therapy	End date of therapy	# Exposures*	Product type	Brand name	Total dose received, IU/kg	# Days hospitalized for this episode
				—select—			
				—select—			
				—select—			
				—select—			
				—select—			

\* An exposure is defined as a 24-hour period in which FVIII / IX containing product is given to a patient. (Blanchette, VS et al. 2014)

## national patient registry / *registre nacional de patients*

Organizations and hemophilia treatment centres are key partners in this network. They play a major role in improving care within their countries.

Starting on the path to improved care can be a daunting task. An important early step is to establish a national patient registry. A patient registry is an invaluable tool for improving the lives of people with hemophilia. It is essential for tracking the identification and diagnosis of people with hemophilia, and monitoring of their health, as well as long-term planning for hemophilia organizations, and priority setting for health care. Having a national patient registry means being able to answer fundamental questions about the hemophilia population within your country – such information is essential for lobbying government and advocating for improved care.

In a joint meeting of the World Health Organization (WHO) and WFH on Hemophilia Care in Developing Countries, held in Geneva, Switzerland in 1997





invaluable tool for improving the lives of people with haemophilia:  
*eina valuosa per millorar la vida de les persones amb hemofilia*

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## monitoring of their health / *monitoritzar la seva salut*

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## long term planning / *planificació a llarg termini*

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priority setting for health care / *establiment de prioritats per a la cura de la salut*

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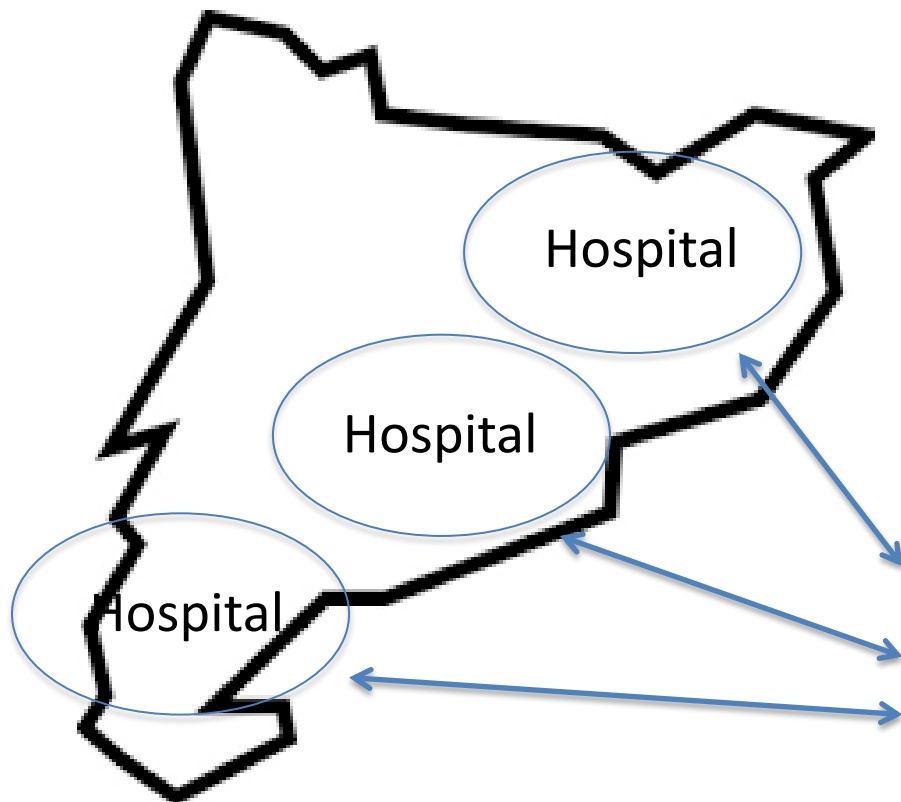
to answer fundamental questions about the hemophilia population  
*/ respondre preguntes fonamentals sobre la població hemofílica*

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**CatSalut**

- Base de dades
- Coordinació / Data Manager
- Control de Qualitat

## REGISTRE: UTILITAT

### Coneixement



- Conèixer el **cens real** de pacients amb coagulopaties congènites. Distribució geogràfica, per patologies, per gravetat,...
- Conèixer **el resultat en salut**. Complicacions
- Conèixer els resultats reals **d'eficiència dels fàrmacs**
- Vetllar per la **seguretat** a llarg termini
- Millorar el coneixement de la **gestió de la malaltia** a partir dels informes/dictàmens
- Dissenyar **estudis** observacionals
- Conèixer el **cost total** del tractament de les diferents patologies

## REGISTRE: UTILITAT

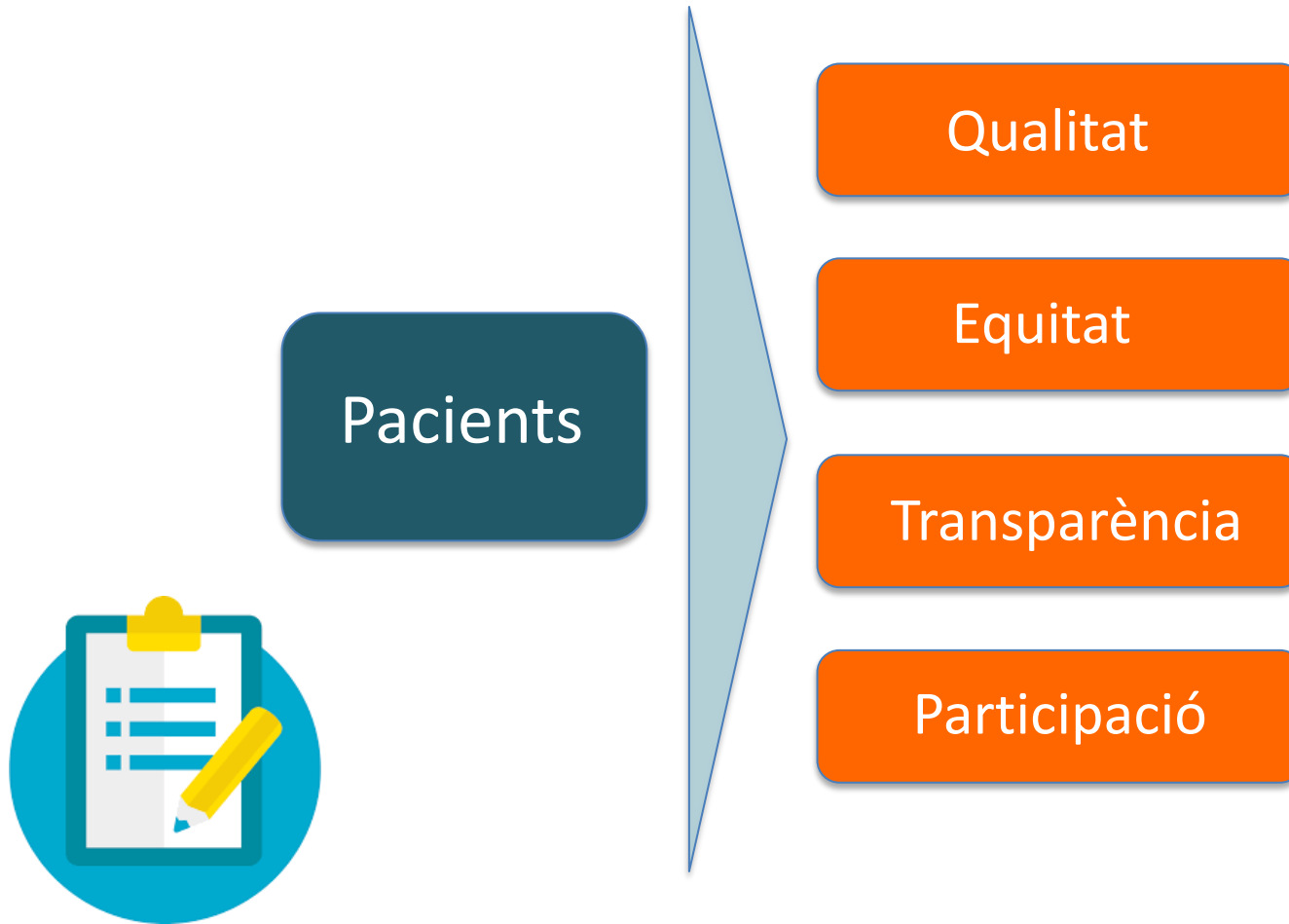
**Suport a la  
planificació  
i presa de  
decisiones**



- Ajudar a la **presa de decisions** enfront d'un pacient
- Focalitzar la **recerca**
- Millorar la **planificació i organització de recursos**
- Reforçar **l'adequació i l'eficiència** dels tractaments
- Establir **protocols** de tractament
- Disposar d'informació per a la planificació, seguiment i disseny de **pressupost**
- Millorar en la **presa de decisions**



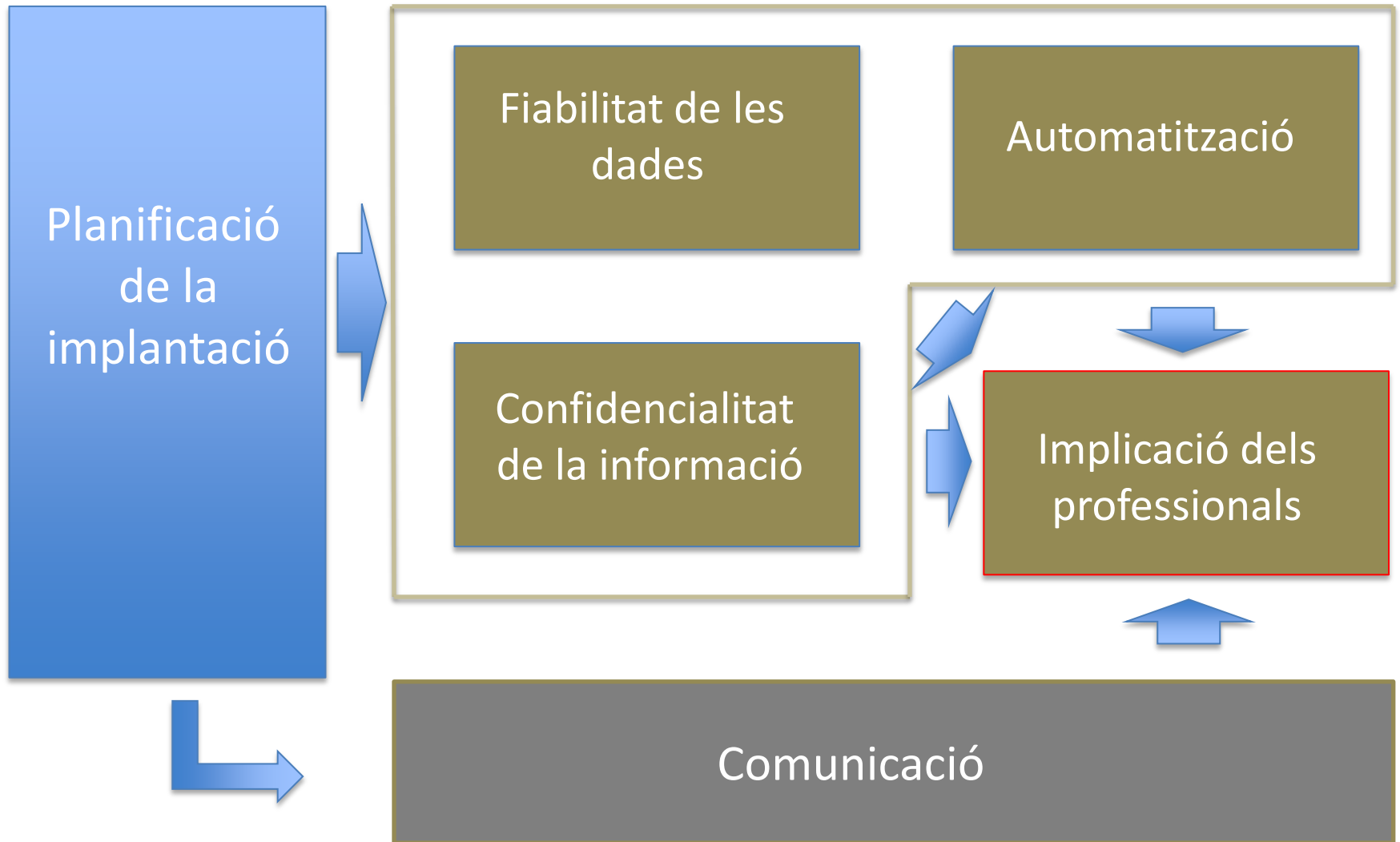
## REGISTRE: IMPLICACIONS PEL PACIENT



# REGISTRE: FACTORS D'ÈXIT

Confiança

Facilitat dús



## 2. GRUP DE TREBALL PER LA REHABILITACIÓ EN L'ARTROPATIA HEMOFÍLICA



**Hemorragia articular  
repetida**



**Artropatia hemofílica**



Mobilitat articular reduïda  
Contractures  
Discapacitat musculoesquelètica  
Dolor



Tractament profilàctic  
amb FVIII/FIX

Noves molècules

Teràpia gènica



**REHABILITACIÓ  
FISIOTERÀPIA**

# EFICÀCIA DE LA REHABILITACIÓ/FISIOTERÀPIA EN LA MOBILITAT, FUNCIÓ ARTICULAR I DOLOR EN PERSONES AMB ARTROPATIA HEMOFÍLICA<sup>1</sup>



- La pèrdua de mobilitat i funció articular s'acompanya de dolor crònic
- **30-50%** persones amb hemofília viuen amb **dolor crònic**
- Principal recomanació: **tractament farmacològic**
- **50-80%** persones amb hemofília **eviten l'exercici físic** pel dolor + artropatia
- La capacitat per moure's és un element essencial de la salut i del benestar

<sup>1</sup>:Recent advances in musculoskeletal physiotherapy for haemophilia. Ther Adv Hematol 2018

## EFICÀCIA DE L'EXERCICI (Revisió Cochrane<sup>1</sup>)

Exercicis de resistència  
Exercicis isomètrics  
Bicicleta estàtica  
Caminar sobre cinta  
Hidroteràpia



Millora del dolor  
Més mobilitat articular  
Més força  
Caminar més estona



Hidroteràpia

Eficaç per disminuir el dolor  
(millor que exercicis en terra)



Exercicis funcionals (cinta, pes,..) millor que exercicis estàtics per millorar la força muscular

<sup>1</sup>: Strike K et al. Cochrane Database Syst Rev 2016; CD011180

## TERÀPIA MANUAL<sup>1</sup>



Eficaç per millorar el dolor  
Millor qualitat de vida  
NO efecte sobre mobilitat o funció articular

## TERÀPIA ELECTROMAGNETICA<sup>2</sup>



RCT. 48 adults. Millora significativa de la funció articular si s'acompanya d'exercicis de resistència

- Exercicis resistència + Teràpia EMG placebo
- Exercicis resistència + Teràpia EMG
- > Teràpia EMG  
No tractament

## TERÀPIA LASER D'ALTA INTENSITAT<sup>3</sup>



RCT. 31 nens amb artropatia precoç.  
Eficaç amb fisioteràpia estàndard, amb millora del dolor i distància al caminar.

<sup>1</sup>: Cuesta-Barriuso R. Physiother Theory Pract 2014

<sup>2</sup>: Parhampour B. Clin Rehabil 2014

<sup>3</sup>: El-Shamy SM. Disabil Rehabil 2018

# RCT – subjective physical performance and quality of life after a 6-month programmed sports therapy (PST) in patients with haemophilia

B. RUNKEL,\* S. VON MACKENSEN† and T. HILBERG\*

\*Department of Sports Medicine, University of Wuppertal, Wuppertal, Germany; and †Institute of Medical Psychology, University Medical Centre Hamburg-Eppendorf, Hamburg, Germany

Estudi randomitzat 64 pacients amb hemofília greu-moderada. Grup control

Programa d'entrenament individualitzat (6 mesos). Fitness center.

Exercicis de mobilitat, coordinació, força progressiva i resistència

## **Millora significativa en:**

Força muscular

Distància recorreguda en 12 min

Temps en equilibri sobre una cama

Qualitat de vida: Salut general, salut mental, estat d'ànim, treball, família i resistència.

PERÒ sense canvis en l'avaluació física articular (escala de Gilbert)



Exercici físic com part de l'estratègia per alleujar el dolor i millorar la força, mobilitat i funcionalitat articular.  
Hidroteràpia i exercici funcional.

Grup expert:

**Dra Almudena Crespo.** Metgessa rehabilitadora. Hospital Vall d'Hebron.

**Dra Carmen Ruber.** Metgessa rehabilitadora. Hospital Santa Creu i Sant Pau.

**Dra Natalia Rodríguez.** Metgessa rehabilitadora. Hospital Sant Joan de Déu.