

Audit: Our Lady's Children's Hospital

Date of Audit: 25th March 2014

Name And Address of Audited Centre:

National Paediatric Haemophilia Centre
Our Lady's Children's Hospital,
Crumlin
Dublin 12

Director(s):

Dr Beatrice Nolan
Consultant Haematologist

Number of Consultants:

Dr Corrina Mc Mahon
Prof Owen Smith
Dr Aengus O'Marcaigh
Dr Melanie Cotter / Dr Andrea Malone (job share)

Nursing Staff (Bands/Job Title/WTEs):

Ms Dympna Cawley, Assistant Director of Nursing	1.0 WTE
Ms Mary Kavanagh, Community Liaison / Haemophilia Nurse Specialist	1.0 WTE
Ms Imelda Kelly, Community Liaison / Haemophilia Nurse Specialist	1.0 WTE
Ms Eibhlin Mc Laughlin, Community Liaison / Haemophilia Nurse Specialist	0.89 WTE
Ms Bridin Brady, Pharmacovigilance Officer / Clinical Nurse Specialist	1.0 WTE

Physiotherapist:

Diarmuid O' Riain

Supporting Staff [Data Manager/Secretary]:

Emma Murphy –Medical Secretary
No data manager

Audit Team

Medical Auditor: Dr David Perry

ADDRESS: Cambridge Haemophilia Centre, Addenbrooke's NHS Trust, Hills Road,
Cambridge, Cambridgeshire, CB2 2QQ

Email: david.perry@addenbrookes.nhs.uk

Nursing Auditor: Ms. Christine Harrington

ADDRESS: Haemophilia Centre, Royal Free Hospital, Pond Street, London, NW3 2QG

Email: c.harrington@nhs.net

Patient/Parent/Carer Auditor[s]: Mr. David Page

ADDRESS: National Executive Director, Canadian Haemophilia Society

Email: dpage@hemophilia.ca

Date of Audit Visit: 25/013/14

Date of Submission of Draft Audit Report To Centre: 13th June 2014

Date of Submission of Finalised Report: 1st August 2014

Audit Standards

A series of standards are provided and against which the Centre should be audited. Free text boxes are available at the end of each section for comments.

The audit document is divided into 5 sections:

- 1. Medical/Nursing/Patient-Parent**
- 2. Medical/Nursing Section**
- 3. Patient-Parent Section**
- 4. Genetics Section**
- 5. Summary**

The sections should be combined at the end of the audit to generate the final audit document

Satisfactory:	Meets minimal acceptable standards
Unsatisfactory:	Falls below minimal acceptable standards

Areas which the auditors consider to be outstanding can be highlighted in the free text boxes. Areas of Best Practice can be summarised at the end of the audit document.

Part 1: Medical-Nursing/Patient-Parent Audit Component

1.1 The Haemophilia Centre

The audit team should note the location, layout, adjacencies etc. of the Centre and a full description of the Centre should be included in the audit report. This information may be provided in advance of the audit by the Centre.

Our Lady's Children's Hospital Crumlin (OLCHC) is an acute paediatric teaching hospital with 227 beds, employing over 1,614 staff. It is Ireland's largest paediatric hospital and is responsible for the provision of the majority of tertiary care service for children including cardiology and cardiac surgery, haemophilia and allied bleeding disorders, haemoglobinopathy, malignant haematology /oncology, stem cell transplantation, burns, medical genetics and medical research for childhood illnesses. The only two children's intensive care units in the country are housed at OLCHC and Children's University Hospital, Temple Street, Dublin.

OLCHC is involved in the teaching of all disciplines of healthcare staff. Undergraduate medical students from University College Dublin, The Royal College of Surgeons in Ireland and Trinity College Dublin receive their training in paediatric medicine at OLCHC.

The hub of the Centre of Children's Nurse Education is located in OLCHC with satellites in Children's University Hospital, Temple Street, Dublin and the National Children's Hospital, Tallaght, Dublin. The Centre of Children's Nurse Education provides continuing education for children's nurses and appropriate staff in the three children's hospitals, the Dublin catchment area and specialist programmes related to children's nursing nationally.

OLCHC is committed to providing family-centred health care in a compassionate and supportive environment, where each child receives the highest standards of health care (OLCHC, Mission Statement).

Philosophy of nursing care in OLCHC

OLCHC is proud to embrace the concept of family-centred care and is holistic and supportive of the role that parents, guardians and families play while their child is in our care. As innovators in the care and management of child health, we, as an organisation, strive continuously to improve the health of children through the integration of care, education and research while:

Providing a child-focussed service with a commitment to children, which respects the uniqueness, individuality and dignity of each child and their family.

Providing excellent standards in the provision of specialist nursing care to children and their families

Sharing the knowledge and expertise gained in the management of these children and their families.

Continuing to champion and advocate for children and their families

Embedding the family centred care ethos into core values and care planning processes within the organisation

The National Paediatric Haemophilia Service has been based at OLGHC since 2004. All elective and emergency surgery for all children with inherited bleeding disorders in Ireland is performed at OLGHC. Clintech, an electronic patient record for patients with bleeding disorders, is available in all clinical areas where children with bleeding disorders are treated, including the Emergency Department.

The service is managed in four areas:

- Haematology/oncology day unit: Elective and emergency day admissions - 13 beds.
- Emergency department: Out-of-hours emergency review and admissions
- St. Michaels & Nazareth ward: In-patient admissions

Neonates and infants are admitted to Nazareth ward

Older children are admitted to St Michael's ward

- HOOPS clinic: Outpatient clinics
 - Haemophilia multidisciplinary clinic
 - Haemostasis and Thrombosis clinic
 - Transition clinic
 - Nurse led clinic
 - Prophylaxis clinic
 - Vaccine clinic
- The Haemophilia Clinical Programme Quality Manual was available for review on the day of the audit.
- The centre has recently submitted an application to EUHANET for European Haemophilia Comprehensive Care Centre certification.

1.2 Coagulation Factor Stock Control, Storage & Issue

Audit Standard: CCCs should have in place adequate procedures for factor concentrate ordering, storage, stock control, recording of issue to patients and their use by patients.

1. Procedures for the ordering of factor concentrate

Coagulation factor concentrates (CFCs) are ordered by the Blood Transfusion Laboratory from Temperature Controlled Pharmaceuticals (TCP) (SOP:LP-BTS-BldOrderSel). A completed Blood Transfusion Request Form specifying name and dose of CFC required is sent to the Blood Transfusion Laboratory. The Laboratory Information System contains the transfusion records of all patients attending OLCHC. The record of each child with a bleeding disorder contains the details of CFC assigned for treatment.

A list of patients with inhibitors and their current treatment is produced monthly by the Coagulation Laboratory and a copy given to the Blood Transfusion Laboratory. This list is checked against the individual patient's flags and if necessary changes are made (LP-BTSFactorDef). Refer to Guidelines for the Administration of Coagulation Factor Concentrates, CP-H&TCFCGuide.02

Satisfactory

~~Unsatisfactory~~

2. Facilities for the storage of concentrate

Stocks of CFC are stored in Stock Fridge in the Blood Transfusion Laboratory at 2-6°C (SOP: LP-BTS-StkEntry). Issued CFCs are stored in 3 designated "issue" fridges: 1) in the Blood Transfusion Laboratory, 2) in the theatre area 3) Haematology/Oncology Day unit. These fridges are fitted with a continuous temperature monitoring system (SOP:LP-BTSRees). All fridges are temperature mapped and have a preventative maintenance schedule with 2 PMs annually (SOP: LP-BTS-Frifre)). Refer to Guidelines for the Administration of Coagulation Factor Concentrates, CP-H&TCFCGuide.02

Satisfactory

~~Unsatisfactory~~

3. Procedures for stock control

CFC stock is managed by the Blood Transfusion Laboratory (SOP LP-BTS-ManStock). Minimum stock levels are set at which stock is replenished. All stock is recorded on the laboratory information system. If a patient is scheduled to receive CFCs over a number of days, the Pharmacovigilance Officer for CFCs (PO) informs Blood Transfusion Laboratory staff and a written request is sent to the laboratory in advance thus allowing the laboratory maintain minimum stock levels, and still meet the patients' requirements.

Satisfactory

~~Unsatisfactory~~

4. Procedures for recording of concentrate issues to patients

All CFCs are issued from the Blood Transfusion Laboratory on a named patient basis. When the CFC is issued to a patient the laboratory system allocates an ID number for each vial issued, which is recorded manually and electronically on the patient record. Each vial of issued CFC is labelled for that patient. The label contains a portion with the CFC details for the prescription sheet and a 'traceability' tag. This tag is removed and signed when the patient receives the CFC and is returned to the Blood Transfusion Laboratory as proof of infusion. The patient's laboratory records are updated from this tag. If a tag is not returned to the laboratory the PO traces that product to the patient's chart to confirm its fate. Refer to Guidelines for the Administration of Coagulation Factor Concentrates and Managing Adverse Coagulation Factor Concentrate (CFC) Events and Near Misses.

The PO carries out a random vertical audit of CFCs administered throughout the year, from the sign out procedure through to administration, identifying types of incidents by root cause analysis and implementing corrective action to prevent recurrence.

Satisfactory

~~Unsatisfactory~~

5. If home delivery service in place, adequate recording of concentrate issuing by company

All concentrates issued by Temperature Controlled Pharmaceuticals (TCP), the home treatment delivery company, are electronically tracked, traced and recorded using bar code technology.

Satisfactory

~~Unsatisfactory~~

6. Procedures for recording concentrate usage by patients on home treatment (e.g. Haemtrack, paper records] and documented evidence that this is being undertaken

Parents record CFC usage and batch numbers either manually or electronically. The paper record is sent to the data manager at NCHCD, St James's Hospital who records CFC usage on the Clintech system.

There are 24 patients electronically recording CFC home treatment. 16 are using the Home Scan Application (App) and the remainder are using the HTC Hand Held Device. Both prophylaxis and on-demand treatments are electronically recorded. Each time a patient selects the on-demand treatment option, they receive an alert notice on the phone advising them to contact their Treatment Centre. We also receive an alert email indicating the bleed site. Upon reading the e-mail the CNS in Haemophilia or the PO will contact the parent if the parent has not already contacted the hospital about the bleed episode.

The Home Scan Website is audited on a monthly basis by the PO to determine compliance in scanning Home Treatment. At each clinic visit the PO meets with patients/parents using the Home Scan App or HTC Hand Held Device to determine whether they have any issues. In addition the Home Scan Website can generate a report listing CFCs administered for prophylaxis and bleeding episodes. We can determine if the prescribed prophylaxis and correct doses for treatment of bleeds has been administered.

Advantages of electronic recording:

- It provides safety checks on the product: correct product, expiry date & recall list
- It's a user friendly system
- Provides accurate and real-time data to the clinicians
- Built in alerts for high risk bleeds
- Cost saving, as it allows us to compare despatches versus usage and by having real-time data, we can intervene where there are breakthrough bleed issues.

Disadvantages - these are mainly technical issues associated with the HTC hand held devices and include:

- Scanning problems
- Failure of application to sync with database.

“Medication recording in the home to improve patient safety ...there’s an App for that”

This project was a collaborative process between the National Centre for Hereditary Coagulation Disorders, St James’s Hospital, Cork Comprehensive Coagulation Centre and OLCHC. It is presently being implemented in OLCHC by Bridin Brady. (PO) The project received a Commendation in the Information Technology Category of the Irish Healthcare Awards 2013.

Comments:

No major problems were identified.

There is a clear mechanism in place for the ordering of clotting factor concentrate and each batch can be tracked to a specific patient. There is a mechanism in place [paper and electronic] for the recording of treatments and bleeds.

There was some concern about the necessity for double bar-coding concentrate vials but this has been and was vigorously defended.

1.3 Treatment

Audit Standard: Patients who are actively bleeding receive prompt and effective treatment according to established protocols throughout the 24-hour period. Appropriate arrangements are in place for routine patient review and liaison with local haemophilia centres for shared-care patients. Adequate mechanisms and protocols are in place for home treatment, prophylactic administration of concentrate and management of inhibitor patients where appropriate. General and genetic counselling is readily available for patients and their families.

1. There is an appropriate treatment area that provides privacy and comfort

Satisfactory [see comments] Unsatisfactory

2. Universal cross-infection precautions are in place

Satisfactory Unsatisfactory

3. There is effective recording of patients' vCJD at risk status and appropriate health care measures are in place for 'at risk' patients.

There are currently no patients in OLCHC on the "at risk register" for vCJD

Satisfactory Unsatisfactory

4. There is evidence of regular review of patients

Satisfactory Unsatisfactory

5. There is in place a formalised pathway protocol for out-of-hours patient review and care

Accessing Inpatient Services CP-H&T-AIS.02 describes the pathway for out of hours care.

Satisfactory Unsatisfactory

6. Consultant haemophilia medical staff is available 24 hours a day for treatment advice.

A haematology registrar and haematology consultant are on-call at all times.

Satisfactory Unsatisfactory

7. There is evidence of effective community liaison between the unit and the patient in their home and with primary care providers

Haemophilia Outreach Nursing Service CP-H&T-HONS.01 outlines community liaison Procedure for taking and recording phone calls within the Haemophilia Service CP-H&T-PHONE.01

Satisfactory

Unsatisfactory

8. There is evidence of treatment in the community by nursing staff [This may be not available in some/many centres]

Haemophilia Outreach Nursing Service CP-H&T-HONS.01 outlines community liaison

Satisfactory

Unsatisfactory

It is difficult to see that the community outreach service can be sustained unless there is a consistent establishment of at least 3 WTE haemophilia nurses.

9. Written protocols/guidelines/procedures are available for the following:

	Satisfactory	Unsatisfactory	N/A
1. Management of bleeding episodes	Yes		
2. Commencement of home treatment/Venous access training	Yes		
3. Commencement of prophylaxis in children	Yes		
4. Management of inhibitor patients (including immune tolerance)	Yes		
5. Treatment in the Emergency Department	Yes		
6. Management of pregnancy/delivery			NA
7. Genetic counselling	Yes		
8. Management of surgery	Yes		
9. Transitional care	Yes		

All OLCCH Policies, Procedures and Guidelines were available for viewing on day of audit.

10. Detail the mechanisms in place for orientation of medical/nursing staff for procedural training.

Please see the Haemophilia Clinical Programme Quality Manual for details of staff training.

Comments: This was discussed during the audit and appeared satisfactory.

Comments including any that relate to feedback from regional Haemophilia Centres

None received but this was not part of this audit – however see patient feedback later in this audit document.

There is a clear and robust system in place but we felt that the day ward used to treat patients lacked privacy and this is something that the staff may wish to explore and to audit to generate objective data. Patients needing infusions expressed a strong preference to be seen in the out-patient clinic where they have their regular assessments.

There is a robust quality management system but with the retirement of the quality manager shortly, it is not clear how this will continue. At present there is close integration with St James's Hospital and their quality manager.

The breadth of guidelines and protocols was impressive and these were widely available.

There is an increasing incorporation of 'service users' into service development and this should be encouraged.

1.5 Age Appropriate Treatment Facilities and Services – If Relevant

	Satisfactory	Unsatisfactory
Appropriate paediatric in-patient and out-patient facilities	Yes	
Child friendly waiting/play area and toys	Yes	
Child friendly treatment area	Yes	
Out-of-hours treatment facilities	Yes	
Paediatric resuscitation facilities	Yes	
Training in paediatric resuscitation	Yes	
Use of local anaesthetic creams and distraction techniques	Yes	
Appropriate transitional arrangements for the transfer of adolescents to adult services are in place	Yes	
Effective outcome monitoring of patients on prophylaxis	Yes	

COMMENTS

No problems were identified.

There is discussion in place looking at the transition from a paediatric setting to an adult setting and how this is best achieved aiming to ensure a smooth and trouble free pathway.

There are weekly MDT meetings.

1.6 Availability Of Comprehensive Care Services

Audit Standard: Services required to provide a comprehensive care service are available as detailed in the Haemophilia Alliance National Service Specification. This will be replaced in 2013/2014 by the Clinical Reference Group [CRG] Specification.

A full description of these should be included in the free text audit report.

The following services/personnel are available;

	Yes	No
Centre receptionist/secretary	Ms. Emma Murphy	
Centre data/business manager		√
Access to a social worker	Ms Carol Carr	
Dedicated Physiotherapist	Mr. Diarmuid O' Riain	
Access to a Psychologist/Counsellor	Dr Yvonne Duane Principal clinical psychologist *part time*	
Dental service/Dentist (a full description of the service offered should be included)	Dr Kirsten FitzGerald Dr Paddy Fleming	
Orthopaedic service/Orthopaedic surgeon	√	
General/Specialist surgical services	Mr Brice Antao	
HIV physician	√	
Hepatologist	√	
Obstetric/Gynaecology service/Surgeon	Adolescent gynaecology service at AMNCH, Tallaght, Dublin Obstetric service – referral made to Haemophilia Service, St James's	

	Hospital , which then liaises with appropriate obstetric service	
Paediatrician (in paediatric or paediatric/adult centre)	√	
Dietician	√	
Genetic Counselling Services [Provide a description of the service and how this is accessed]	√	
Antenatal diagnosis arrangements		NA
Home Delivery [Comments]	√	
Access to Occupational Therapy	√	
Access to Benefits Advice	√	

COMMENTS:

Job description SOP has been attached as supporting evidence

Comments:

(Any contacts made with multidisciplinary staff during the visit should be noted)

1. Community dental services are problematic although the Centre provides a series of information leaflets for both patients/families and dental practitioners in an attempt to alleviate the inevitable anxieties. Most children are well looked after in the community. Within the hospital there are long waiting times for dental surgery e.g. 4-6 months. This is unacceptable for the management of dental problems in children. There is only a single dental nurse assisting up to 4 dental practitioners within the hospital and again this is not ideal and a further dental nurse should be appointed to assist in this area.
2. Social worker: A social worker keeps in contact with newly diagnosed children/families and this was seen as invaluable.
3. The current physiotherapist covers both malignant and non-malignant haematology. The physiotherapist attends the weekly haemophilia clinic and the weekly MDT meetings. Every child seen on the day ward by the physiotherapist is also seen by a haematology doctor. The physiotherapist service appears to run well.

1.7 Patient Choice & Experience

1. How you been able to access information about your local CCC that allows you compare the services it offers with other CCCs?

2. Are there any Patient Satisfaction Survey/Audits/Focus Groups Reports available?

- The National Home Treatment Satisfaction Survey is currently in progress. The questionnaire will be available for viewing with any completed questionnaires
- Satisfaction Survey on Vein Training completed in 2012. The report was available for viewing on day of audit
- Satisfaction Survey on CVAD Training completed in 2012. The report was available for viewing on day of audit
- Haemophilia Nurse Led Clinic Satisfaction Survey completed in 2013. The report was available for viewing on day of audit
- DDAVP Education satisfaction survey completed in 2013. The report was available for viewing on day of audit
- The OLCHC haemophilia team meets with the Irish Haemophilia Society on a quarterly basis. The meetings allow the patient group to raise issues that impact the care of haemophilia patients nationally and to improve haemophilia care and the patient's experience.
- Representatives from the OLCHC haemophilia team attend the Irish Haemophilia Society AGM
- The OLCHC haemophilia team collaborated with the Irish Haemophilia Society in setting up an annual Parents/Family Weekend. Representatives from OLCHC haemophilia team attend and are available to parents and children for discussion of all issues related to their care and to the OLCHC haemophilia service
- OLCHC have devised Patient Feedback forms. A copy was available for viewing on day of audit
- Two informal patient focus groups were held in 2013 in order for parents to meet each other and offer any suggestions for improving the service to the team
- Parent satisfaction survey is currently being developed for outpatients. The questionnaire will be completed on an IPAD.

Comments:

With the exception of the parents' desires not to have their children receive infusions in the day ward, comments were extremely positive. This one sums it up: *'The support given to my family from the whole team is beyond what is required of them. We are grateful for this; they offer care in all aspects of life.'*

1.8 Clinical Governance, Audit, Teaching, CPD & Research

Audit Standard: There is evidence that CCC staff participate in clinical governance, audit and teaching activities. There is evidence that unit staff undergo regular personal performance review and participate in CPD schemes. The unit participates in clinical trials and active research.

1. CCC staff participate in clinical governance and audit activities?

YES NO

2.1 List audits performed in the last 3 years

Please see the audit folder on day of audit

2.2 Discuss the changes in practice that have arisen from these audits and how these are monitored?

Please see the audit folder for all outcomes and changes in practice arising from audit.

3. CCC staff participate in teaching?

YES NO

Give examples of teaching activities:

LABORATORY

All team members participate in twice monthly Haematology/Oncology Grand Rounds
The Laboratory is a designated training laboratory for undergraduate Medical Scientists in association with the Dublin Institute of Technology and Trinity College, Dublin.
Staff members present at national conferences/courses.

For example:

National Paediatric Haemophilia Study Day
Academy of Medical Laboratory Sciences

NURSING

Organisational Workshops

- Haemophilia & bleeding disorders
- Surgery in the child with haemophilia
- DDAVP / DDAVP Trial
- Coagulation factor concentrates
- Factor calculations/reconstitution/administration
- Port-a-caths

Organisational Lectures

- Haemophilia & bleeding disorders
- Surgery in the child with haemophilia
- Coagulation factor concentrate
- Port-a-caths
- Child Protection Awareness Training
-

External Education

- BNS students University College Dublin
- Medical students RCSI
- Irish Haemophilia Society
- Irish Haemophilia Society Parents' Weekend
- Irish Haemophilia Nursing Study Day
- Barretstown Gang Camp (Hole in the Wall Foundation)
- School lectures
- PEP programme (Parents Empowering Parents)

MEDICAL

Undergraduate medical students of Trinity College Dublin, University College Dublin and Royal College of Surgeons of Ireland (attachments and lectures)

Postgraduate medical/ paediatric:

- paediatric specialist registrars, RCPI Faculty of Paediatrics
- haematology specialist registrars, RCPI Faculty of Pathology and ICHMT
- OLVHC paediatric staff

Postgraduate nursing:

- Paediatric ICU Nurses
Paediatric Haematology/ Oncology Nurse Course

Postgraduate Medical Laboratory Science courses

Parents/ Patients

Irish Haemophilia Society parents' weekend
Irish Haemophilia Society AGM
Irish Haemophilia Society family weekend

Miscellaneous

Hospital Grand Rounds
Haematology/ Oncology Grand Rounds
Haemophilia Journal club
National Paediatric Haemophilia Conference
National Haemophilia Conference (biennial)

DENTAL

- Undergraduate dental students at the Dublin Dental University Hospital, Trinity College
- Postgraduate dental students at the Dublin Dental University Hospital, Trinity College

Dr Kirsten FitzGerald, Consultant Paediatric Dental Surgeon will attend the World Federation of Haemophilia in Melbourne as an invited speaker. She is a Consultant to the American Board of Pediatric Dentistry for their Oral Clinical Examination

Dr Paddy Fleming, Consultant Paediatric Dental Surgeon, is the President elect of the European Academy of Paediatric Dentistry, The Academy is involved in promoting high standards in education, practice and research in all aspects of paediatric dentistry in Europe

PHYSIOTHERAPY

- In-house in service training for NCHDs, Nursing and MDT
- Partake in fortnightly Haematology/Oncology Grand Rounds
- Participation at Irish Haemophilia Society Family Weekend
- Lectures to Paediatric Physiotherapy special interest group on the role of Physiotherapy in Haemophilia
- Lecture at National Paediatric Haemophilia Conference OLCHC
- Annual performance review is in the form of a one to one Personal Development Interview with the Physiotherapy manager discussing ongoing performance, mandatory training, education and research opportunities.

4. CCC staff undergo regular performance review?

Medical and laboratory staff undergo objective setting and review.

Nursing: CNS submits quarterly progress report to the Assistant Director of Nursing. Personal and professional development plans exist through nursing service planning for 2014

Centre of Children's Nurse Education training records for mandatory days

Physiotherapy: Annual performance review is in the form of a one to one Personal Development Interview with the Physiotherapy manager discussing ongoing performance, mandatory training, education and research opportunities.

YES NO

5. CCC staff participate in continuing professional development?

Attendance lists of all national and international conferences are available for viewing on day of audit

YES NO

6. The unit participates in clinical trials:

Study of Recombinant Coagulation Factor VIII Fc Fusion Protein, BII031, in Pediatric PTP Subjects with Haemophilia A

Sponsor: Biogen Idec

ClinicalTrials.gov identifier: NCT01458106

EudraCT number: 2011-003073-28

Long- Term Safety and Efficacy of Recombinant Human Coagulation Factor VIII Fusion Protein (r FVIII Fc) in the Prevention and Treatment of Bleeding Episodes in Previously Treated Subjects with Hemophilia A

Sponsor: Biogen Idec

ClinicalTrials.gov identifier: NCT 01454739

EudraCT number: 2011-003072-37

Study of Recombinant Coagulation Factor IX Fc Fusion Protein, BII031, in Pediatric PTP Subjects with Haemophilia B

Sponsor: Biogen Idec

ClinicalTrials.gov identifier: NCT 01440946

EudraCT number: 2011-003076-36

Long- Term Safety and Efficacy of Recombinant Human Coagulation Factor IX Fusion Protein (r FIX Fc) in the Prevention and Treatment of Bleeding Episodes in Previously Treated Subjects with Hemophilia B (B-YOND)

Sponsor: Biogen Idec

ClinicalTrials.gov identifier: NCT 01425723

EudraCT number: 2011-003075-11

A multi- centre phase III uncontrolled open- label trial to evaluate safety and efficacy of BAY81- 8973 in children with severe haemophilia A under prophylaxis therapy

Sponsor: Bayer

ClinicalTrials.gov identifier: NCT 01311648

EudraCT number: 2010-021781-29

PROPACT: Retrospective Prophylaxis Patient Case Collection

Sponsor: Novo Nordisk

ClinicalTrials.gov identifier: NCT 00882778

Observational Study on Safety of Room Temperature Stable NovoSeven in Patients with Haemophilia A or B

Sponsor: Novo Nordisk

ClinicalTrials.gov identifier: NCT 01220141

EudraCT number: N/A

Currently with Ethics Committee

Sponsor: Biogen Idec

Full title of study: An Open-Label, Multicenter Evaluation of the Safety and Efficacy of Recombinant Coagulation Factor IX Fc Fusion Protein (rFIX Fc; BII029) in the Prevention and Treatment of Bleeding in Previously Untreated Patients with Severe Hemophilia B

Protocol number: 998HB303

EudraCT number: 2013-003629-27

Also under consideration / pending

Sponsor: Biogen Idec

Full title of study: An Open-Label, Multicenter Evaluation of the Safety and Efficacy of Recombinant Coagulation Factor VIII Fc Fusion Protein (rFVIII-Fc; BIIIB031) in the Prevention and Treatment of Bleeding in Previously Untreated Patients with Severe Hemophilia A

Protocol number: 997HA306

EudraCT number: 2013-005512-10

YES

NO

6. The unit participates in clinical research:

OLCHC is a member of PedNet, the European Paediatric Network for Haemophilia Management and contributes data to the PedNet Haemophilia Registry

PedNet and Research of Determinants of Inhibitor development (RODIN) Study Group. Intensity of factor VIII treatment and inhibitor development in children with severe hemophilia A: the RODIN study.

Gouw SC, van den Berg HM, Fischer K, Auerswald G, Carcao M, Chalmers E, Chambost H, Kurnik K, Liesner R, Petrini P, Platouki H, Altisent C, Oldenburg J, Nolan B, Garrido RP, Mancuso ME, Rafowicz A, Williams M, Clausen N, Middel R, Ljung R, van der Bom JG. Blood 2013 May 16; (20): 4046-55

Audit of bleeding complications & cost of dental treatment for children with hereditary bleeding disorders using a new treatment protocol

U. Rao, I. Kelly, M. Kavanagh, B. Brady, K. Fitzgerald, B. Nolan, P. Fleming. Haemophilia (2013), 19 (Suppl. 2), 55

Low risk of inhibitor formation in haemophilia A patients following en masse switch in treatment to a third generation full length plasma and albumin-free recombinant factor VIII product (ADVATE®).

Bacon CL, Singleton E, Brady B, White B, Nolan B, Gilmore RM, Ryan C, Keohane C, Jenkins PV, O'Donnell JS. Haemophilia. 2011 May; 17(3):407-11

Dentists' knowledge and views on treating children with hereditary coagulation disorders
K. Fitzgerald, P. Fleming & B. Nolan.

International Journal of Paediatric Dentistry, page 60. Volume 21, Issue Supplement S1, June 2011

A review of severe factor X deficiency in patients in the Republic of Ireland

Mary Kavanagh, Ann O'Sullivan, Imelda Kelly, Irene Regan, Mary Byrne, Vince Jenkins, Dymrna Cawley, James O'Donnell, Beatrice Nolan · Journal of Thrombosis and Haemostasis , 2011 Volume 9, S 2

The laboratory investigation of inhibitors (alloantibodies) in patients with haemophilia A and B in a National Paediatric Comprehensive Care Centre. Thesis for BSc Biomedical Sciences, DIT/Trinity College Dublin.

Downes E, Philbin B, Regan IE and Nolan B.

YES

NO

COMMENTS:

There is clear evidence of an active research/audit programme to guide and inform practice and the staff should be congratulated on this.

2. The Haemostasis Laboratory

Audit Standard: The haematology laboratory in which the CCC haemostasis laboratory is located should have full CPA accreditation. The haemostasis laboratory should be adequately staffed with an appropriate skill mix and have adequate space and facilities to perform an effective diagnostic and monitoring service. The laboratory should participate in a national quality assurance scheme. Clotting factor assays should be available throughout the 24-hour period.

Patients-Parents-Carers in this part of the audit may wish to explore turnaround times for samples and how urgent results are communicated to the referring doctor.

1. The Haemostasis laboratory has full CPA accreditation:

YES NO

If yes, date of last CPA inspection:

CPA inspection 2012
ISO 15189 2014 (INAB)

2. The staffing levels and skill mix is adequate to provide an effective service:

YES NO

3. The laboratory space and facilities are adequate:

YES NO

4. The laboratory participates in a national quality assurance scheme in coagulation:

NEQAS, ECAT and LabQuality

YES NO

5. Has there been any persistent poor performance over the previous two years?

No

YES NO-NO

If yes, list the problem assays

6. The following tests are available in the haemostasis laboratory:

	YES	NO
All coagulation factor assays	√	
FVIII Inhibitor screening	√	
FVIII Inhibitor quantification	√	
VWF antigen	√	
VWF activity * available in house beginning May 2014	√	
VWF multimers		√ Royal Free Hospital
Platelet function testing	√	
Platelet granular constituents		√ NCHCD

If any of the above tests are not performed outline the alternative testing arrangements.
See above

7. List any diagnostic tests that are performed not listed above:

All factor inhibitors

8. Coagulation factor assays are always available throughout the 24-hour period:

YES NO

9. A diagnostic genetic laboratory service is provided

Referral arrangements with NCHCD

[The Genetics Service will be audited separately during the current audit]

YES NO

COMMENTS

The laboratory provides a high quality service in cramped conditions.
During a recent INAB inspection no major problems were identified.

3. Paediatric Care In Centres Looking After Children

Audit Standard: The care of children with haemophilia and related disorders can be complex and should only be carried out by staff who are experienced and trained in the management of children. Facilities should be adequate for the care of children.

3.1 Staff qualifications

Medical Staff	Yes	No
Consultant Haematologist has paediatric training and expertise	√	
Consultant Paediatric Haematologist	√	
Named Consultant Paediatrician supporting Consultant haematologist (in centres without a Consultant Paediatric Haematologist)	N/A	
Consultant Paediatric Surgeon with experience of implantable venous access devices	√	
All medical staff involved in managing children should have 'Safeguarding Children Level 3'. In OLCHC all staff complete induction training on "Child Protection"	√	
Nursing Staff/Physiotherapist		
Unit nursing staff have appropriate qualification e.g. Registered Sick Children's Nurse(s) (RSCN) RN Child Branch (Project 2000) BA Nursing (Child)	√	
All nursing staff involved in managing children should have 'Safeguarding Children Level 3' In OLCHC all staff complete induction training on "Child Protection"	√	
Physiotherapist [relevant paediatric qualification.	√	

COMMENTS:

No major problems were identified but there are clearly some staffing issues in relation to senior nursing staff with 2 senior nurses about to go on maternity leave. Consideration must be given as to how the service will continue/survive in the absence of key personnel.

3.2 General Paediatric Services

	Satisfactory	Unsatisfactory
Availability of trained/experienced physiotherapists	√	
Growth and development assessment programme	√	
Availability of play therapist	√	
Liaison with Health Visitors/School nurses	√	
Liaison with nurseries and schools	√	

There is also a hospital school. If the child or adolescent is unable to attend the hospital school, the teachers bring schoolwork to the bedside.

OLCHC has the only hospital-based branch of the Girl Guides in Ireland, with a meeting every Thursday evening

Comments:

No problems were identified.

All staff that were met by the auditors were clearly committed to delivering a high quality, child and family focused service.

4. Outcome Measures

Outcome measures are a fundamental part of the revised audit programme. A nationally agreed dashboard for collecting data on individuals with inherited bleeding disorders will come into operation in 2013. In addition there will also be nationally agreed CQUINS. In the current round of audits, evidence should be provided for both data collection/outcome measures and locally agreed CQUINS – if these exist.

The following is a suggestion as to the sort of data that individual centres may be collecting. Evidence of previous CQUINS or Outcomes Measures collected over previous years should be recorded if available.

Audit Standards:

1. Individuals with a severe inherited bleeding disorder should have an Annual Joint Score performed. How has this been obtained and by whom?
2. Individuals with a severe inherited bleeding disorder and on home treatment [either on-demand or prophylaxis] should record their treatments on Haemtrack or on paper. Evidence of this must be provided to the auditors.
3. Individuals with a severe inherited bleeding disorder should have their BMI calculated annually and if raised they should be counselled and if necessary referred for dietary advice and support. Evidence of this must be provided to the auditors.
4. Individuals with severe Haemophilia A or B should have a quality of life survey annually – HAL or PED-HAL [for children >4yrs].

Comments:

1. Annual Joint Scores are obtained and documented on Clintech by the Physiotherapist.
2. Individuals on Home Treatment record their treatments on either their Home Scan App or Pink Treatment Sheets.
3. BMI calculation is currently being applied to the Clintech patient record and once completed will be recorded at the patient's annual review by the clinical nurse specialists.
4. The NCHCD is in the process of applying Quality of Life Assessment tools onto a mobile device. It is planned that the patient will complete the assessment at their clinic appointment. A pilot programme will be completed in each comprehensive care centre prior to roll out.

The following Quality of Life questionnaires will be applied to the mobile device in OLCHC:

EQ5D-5L- applied to the Home Scan App

CHO-KLAT – applied to a clinic IPAD (parents and /or patients questionnaire)

A demonstration of these tools will be provided on day of NCHCD audit

5. Part 2: Patient Medical Records Review [Medical/Nursing Auditors]

Audit Standard: The following should be present in the patient's medical records.
[Note - In some centres a separate genetics/pedigree file may be used.]

- Clear documentation giving the diagnosis and usual treatment
- Genetic mutation
- Family pedigree with identification of obligate carriers/confirmed carriers
- Appropriate review interval as per National Service Specification recommendation (six monthly for severe and moderate haemophilia, yearly for mild)
- Appropriate physiotherapy/orthopaedic referral with evidence of timely referrals/input/review and assessment
- Appropriate management of HIV, hepatitis B/Hepatitis C infection as per national guidelines where applicable
- Evidence of effective communication with primary and secondary care colleagues and affiliated regional haemophilia centres.

A random sample of 8 medical records should be reviewed.

1. There is documentation giving the patient's diagnosis

Number of records with this information: 8

2. There is documentation giving the patient's treatment

Number of records with this information: 8

3.1 vCJD status is recorded for all relevant patients

Number of records with this information: N/A

3.2 There are appropriate health care measures in place for 'at risk' patients

Number of records with this information: N/A

4. There is documentation of the patient's genetic mutation
This is available on Clintech 8

Number of records with this information:

5. There is documentation of the family pedigree 8

Number of records with this information:

6. There is evidence of appropriate follow up review:

Number of records showing this to be satisfactory: 8

7. There is evidence of appropriate physiotherapy/orthopaedic referral

Number of records showing this to be satisfactory: 8

8. There is evidence of regular dental review

Number of records showing this to be satisfactory: 8

9. There is evidence of appropriate management of HIV, HBV, HCV infection where applicable

Number of records showing this to be satisfactory: N/A

10. There is evidence of effective communication with general practitioners and consultant colleagues

Number of records showing this to be satisfactory: 8

11. Investigation results are readily accessible in the medical records or electronically

Number of records showing this to be satisfactory: 8

COMMENTS:

The case notes were acceptable but:

a. There was no front sheet indicating diagnosis/severity and current treatment. This information was available in the notes but needed 'trawling' to find it. In some cases the particular type of treatment was unclear e.g. 'rFVIII concentrate' but it is unclear which FVIII concentrate this referred to. However, we are aware that Clintech is used to provide this information and that Clintech is available in all clinical areas.

b. In some cases two patients were reported on the same letter. We felt for patient confidentiality this was unacceptable even though these were individuals within the same family.

c. There were problems with abbreviations/spelling and these should be addressed.

6. Part 3: Patient-Parent-Carers Audit Component

This section of the audit will be undertaken whilst the case notes review is taking place by the Medical-Nursing auditors

6.1 Patient Services At The Centre

Audit Standard: Patients, family members and carers attending the CCC should have easy access to the centre, adequate facilities whilst waiting, a private counselling area and availability of written information about all aspects of haemophilia and related disorders.

	Adequate	Inadequate
Access by car		√
Designated Centre Car Parking		√
Access by public transport	√	
Disabled access	√	
Direct Emergency Ambulance Access	√	
Signposting to centre	√	
Direct telephone line	√	
Answerphone	√	
e-mail access	????	
Waiting area	√	
Toilets	√	
Disabled toilets	√	
Age Appropriate Waiting Area)	√	
List of up-to-date educational material	√	
Could you find any information about this CCC on the Internet e.g. Google?	No	
Seating/wheel chair waiting area	√	
Family friendly facilities available	√	

Comments:

Feedback from users:

The auditors received 34 completed questionnaires mailed to a random selection of **Our Lady's Children's Hospital, Crumlin** patients. Of these, 2 were completed by the patients themselves, 27 were completed by the patients' caregivers and 5 were completed for both paediatric and adult patients.

Overall care was rated quite high. 74% (25/34) rated care as excellent. 20% (7/34) rated care as good. 6% (2/34) rated care as average (question 3).

Other results included:

- 97% (31/32) are satisfied with service when ringing the centre for advice (question 4);
- 93% (26/28) are satisfied with the availability of the centre team when needing to be seen for an urgent problem (question 5);
- 96% (25/26) are satisfied with the care they receive when needing to be seen for an urgent problem (question 6);
- 97% (32/33) are satisfied with the arrangements in place for regular check-ups (question 9);
- 100% (33/33) report all their questions are answered to their satisfaction at regular check-ups (question 10);
- 70% (24/34) report they know whom to contact out-of-hours (question 11);
- 56% (19/35) report they have attended an A&E department in the last two years. Of these visits, 74% (14/19) were at Crumlin and 26% (5/19) in other hospitals (question 12);
- 95% (18/19) report they are satisfied with the care they received at A&E (question 13);
- 94% (15/16) are satisfied with the arrangements for home treatment (question 14);
- 100% (11/11) are satisfied with the physiotherapy and orthopaedic services (question 16);
- 97% (30/31) report that dental services are offered through the centre (question 18);
- 76% (13/17) are satisfied with the services for psychological and social support, the principal complaint being that the service was not offered to them (question 21);
- 44% (15/34) are satisfied with access to the centre. Parking was the universal complaint (question 24);
- 6% (2/34) report having ever made a complaint. One of the two complainants reports the complaint was handled to the person's satisfaction (question 25).

Comments about patient experience in the last year

Comments were consistently positive regarding the standard of care, the attention and time devoted by staff, the communication between staff and families and the vein training offered.

Suggestions to improve the centre

These include:

- See the haemophilia nurses in the centre for treatments instead of going to St. John's ward.
- More rooms for treatment in the centre.

- Shorter wait times.
- More availability of the dentist.
- More cooperation between GPs and centre in relation to vaccination service.
- Vaccination appointments in early morning or late afternoon to minimize time missed from school.
- Have a travelling nurse for vein / portacath training.
- Two text messages as reminders for appointments.

Other comments

This is typical of the sentiments expressed: *The support given to my family from the whole team is beyond what is required of them. We are grateful for this; they offer care in all aspects of life.*

Note:

Three questionnaires were received from patients who did not identify with any of the three Irish comprehensive care centres. Limerick and Galway hospitals were mentioned. The respondents rated overall care as either average (1/3), poor (1/3) or very poor (1/3). Telephone advice was unsatisfactory (2/3). Availability of medical care personnel and quality of care for urgent situations was unsatisfactory (2/2). Questions were not satisfactorily answered at check-ups (2/2). A&E care was unsatisfactory (1/1).

(See Annex 1 for all comments.)

7. Part 1 continued: Medical/Nursing/Patient-Parent-Carers Auditors

7.1. Emergency Department/Out-of-Hours Setting:

Audit Standard: There should be a clear pathway for patients with inherited bleeding disorders in the ED. Staff should be aware that individuals with inherited bleeding disorders require to be seen and treated promptly to prevent a minor bleed becoming more serious. Protocols for managing patients with inherited bleeding disorders should be easily accessible. Protocols should be in place for managing 'visitors' who are not known to the CCC.

- SOP on Accessing Inpatient Services CP-H&T-AIS.02 describes the pathway for ED access.

Comments:

There is a clear pathway in place for children attending out-of-hours. Protocols/guidelines are available in the ED. There is an impressive rolling programme of education for ED staff provided by the haemophilia nurses, including reconstitution and administration of clotting factor concentrate. The ED staff met by the auditors all appeared clear about the pathway and guidelines. The user survey results included 18/19 (95%) respondents satisfied with care they received in ED.

7.2 The Haemophilia Service-Genetics Service Interface

1. What proportion of the patients with haemophilia A and B registered at your Haemophilia Centre, have had their causative mutation identified?

All patients with Haemophilia A have had their causative mutations identified

We have been unable to identify the causative mutation in 2 separate kindred with severe factor IX deficiency, and assume the factor IX deficiency is due to large deletions.

2. Do you record this data on the national genetic database (HCIS)?

Yes

No

Comments:

This information is recorded on a database called Progeny which is held in St James's Hospital.

3. Informed Consent: Please describe mechanisms that are in place within your centre to ensure that appropriate informed consent is obtained for genetic testing, and how the laboratory is made aware of any restrictions on consent.

Please indicate which format is used to document patient consent and information regarding testing

- a. UKHCDO consent form and information sheet
- b. UKHCDO consent form and information sheet with local modifications
- c. Alternative format consent form and information sheet (please give details)

Comments:

We use the UKHCDO consent sheet with local modifications.

We investigate probands only.

All possible carriers are referred to the adult service at St James's Hospital for determination of carrier status when they are older than 16 years.

Part 5: Audit Feedback & Closing Meeting

1. The final meeting should include a member/representative of the Trust
2. List the issues raised at the previous audit and indicate whether or not they have been rectified.
3. Highlight areas of best practice.
4. If there are outstanding issues, what are these and what have been the barriers to resolving them?
5. Have any serious issues been identified during the current audit and how will these be addressed?
[In this situation the auditors have a professional responsibility to address these highlight these to the Trust and to the Commissioners. A formal meeting would be convened and the concerns of the auditors discussed.]
6. Issues identified during this audit meeting and mutually agreed provisional plans to address these.
7. If the auditors cannot reach consensus about any aspect of the audit this can be highlighted in the free text boxes below.

Audit Summary: This should highlight areas of best practice and areas that require improvement. The points listed above should form the basis for this summary.

1. We recommend that the pathway for day-care is reviewed.

The haematology day unit is a busy acute setting providing complex care such as chemotherapy for sick children. A number of questions arose in the course of the day that suggested the appropriateness of this setting for children attending with bleeding episodes should be examined. The issues included the use of cannulas rather than butterfly needles for one-off administration of clotting factor concentrates. Vein preservation is a priority for children who will have a life-long requirement for good peripheral venous access. Each visit to the hospital with a young child is a learning opportunity for parents most of whom will eventually manage home infusion using a butterfly needle. Ideally the auditors feel that treatment should be administered by a haemophilia specialist nurse who builds up a working partnership with the child and family.

2. Sustaining haemophilia specialist nursing

A small team of highly specialised nurses is inevitably very vulnerable when there are episodes of maternity or sick leave. Aspects of the service such as community liaison, nurse-led clinics and the adolescent transition programme are difficult to sustain below a minimum of 3 WTE nurses. Should the service be left with less than 3 nurses at any time we would recommend a formal risk assessment of the impact on the service. Consideration could be given to a rotation of nurses from other areas to broaden the base of haemophilia expertise within the hospital that could be a potential source of cover for periods of leave.

Medical Auditor

Comment

There is clear evidence of a high quality, child friendly, patient/family-focused service. There is a philosophy of care and quality throughout the service and indeed the hospital.

There are clearly some staffing issues in relation to senior nursing staff with 2 senior nurses about to go on maternity leave. Consideration must be given as to how the service will continue/survive in the absence of key personnel.

There were some issues in relation to the Day Area and in particular we felt that there was a lack of privacy.

Nursing Auditor

Comment

This is clearly a child and family focused service delivered by a highly motivated team with specialist expertise. There are many aspects of this service which are nurse-led and which suggest that it may benefit from the development of Advanced Practitioner roles.

Patient-Parent Auditor

Comment

Our Lady's Children's Hospital clearly delivers very high-quality care. The staff are well trained and caring. Even out-of-hours Emergency Department care, which is often a problem, is highly appreciated by parents. One parent wrote: *"I would like to thank the haemophilia centre and the IHS for being very good to us for the past 12 years. It was tough going and they were always there for us."*