Alder Hey Children's NHS
NHS Foundation Trust

Performance in Initiating and Delivering Clinical Research

Why are we doing this?

- Through the NIHR (National Institute for Health Research) the Government wishes to see a dramatic and sustained improvement in the performance of providers of NHS services in initiating and delivering clinical research.

- The aim is to increase the number of patients who have the opportunity to participate in research and to enhance the nation's attractiveness as a host for research.

- From 2013 for clinical trials, the NIHR will publish outcomes against contract NIHR benchmarks. Alder Hey holds one of these contracts.

- These outcomes include an initial benchmark of 70 days or less from the time a provider of NHS services receives a valid research application to the time when that provider recruits the first patient for that study ***(Performance in Initiating Clinical Research)***.

- It also includes the NHS providers performance in recruiting to time and target for commercial contract clinical trials ***(Performance in Delivery of Clinical Research)***.

Reporting Period Q3 13/14 – 01/01/2013 to 31/12/2013

Alder Hey Children's NHS
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Performance in Initiating and Delivering Clinical Research

Review of Previous Quarter Data (Q2 01/10/2012 to 30/09/2013)

Comparison of Alder Hey Children's NHS FT against national average

Performance in Initiating

Mean number of days between receipt of Valid Research Application and date of First Patient Recruited = 95 days [SD 88.7]

Alder Hey Children's NHS Foundation Trust = 60.1 days

Rank of Alder Hey for Mean against All Providers 7th out of 52

Total trials meeting the 70 day benchmark = 34.5% of analysed trials

Alder Hey Children's NHS Foundation Trust = 56%

Rank for Alder Hey of % of Trials Meeting Benchmark out of All Providers 10th out of 52

Performance in Delivery

Total closed trials meeting time and target (All Providers) = 16.8%

Alder Hey Children's NHS Foundation Trust = 77.8%

Rank for Alder Hey of Proportion of Closed Trials Recruiting Time and Target 4th out of 52

Reporting Period Q3 13/14 – 01/01/2013 to 31/12/2013

Alder Hey Children's NHS
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NIHR Central Commissioning Facility

70 day benchmark – Time to first patient recruitment

Performance in Initiating Clinical Research

Research Ethics Committee Reference Number	Name of Trial	Date of Receipt of Valid Research Application	Date of First Patient Recruitment	Duration Between VRA and First In	Benchmark Met	Comment
13/NW/0276	European Survey of Paediatric Prolonged, Acute, Convulsive Seizures: Patient and Parent Experience of Current Practice in the Community Setting; Part of the PERFECT Initiative	13/12/13			Within 70 Days	
13/NW/0392	Pilot study on use of Sulphur hexafluoride Microbubbles (SonoVue®) as a Magnetic Resonance Imaging contrast agent in the imaging of brain tumours.	24/11/13			Within 70 Days	
12/NW/0694	A 12 week randomized, openlabel, active comparator period followed by a 12 week safety extension period to evaluate the safety and efficacy of Fesoterodine in subjects aged 6 to 16 years and >25 kg with symptoms of detrusor overactivity associated with a neurological condition (Neurogenic Detrusor Overactivity).	08/11/13			Within 70 Days	
13/NW/0320	A Phase Ib open label, multi-centre study to investigate the pharmacokinetics, pharmacodynamics, and safety of Tocilizumab following subcutaneous administration in patients with Polyarticular-Course Juvenile Idiopathic Arthritis - WA28117	09/07/13	15/07/13	6	Y	
13/LO/0566	A Phase 3, 2-Part, Open-Label Study to Evaluate the Safety, Pharmacokinetics, and Pharmacodynamics of Ivacaftor in Subjects With Cystic Fibrosis Who are 2 Through 5 Years of Age and Have a CFTR Gating Mutation	01/08/13	20/08/13	19	Y	
12/EM/0393	WA28029 - Decreasing TCZ dosing frequency in patients with sJIA	30/04/13	20/05/13	20	Y	
13/NW/0023	Liverpool Pharmacokinetic/pharmacodynamics study of teicoplanin in children (LIPSTIC)	27/03/13	18/04/13	22	Y	
12/NW/0790	BASICS	03/06/13	26/06/13	23	Y	
13/WM/0231	Safety of Nasal Influenza Immunisation in Egg Allergic Children - The SNIFFLE study	01/10/13	04/11/13	34	Y	
12/SW/0380	SPACe Support for Parents with a Child with JIA	07/05/13	19/06/13	43	Y	
11/SC/0543	FOR DMD: Duchenne muscular dystrophy: double blind randomized trial to find optimum steroid regimen	21/01/13	08/03/13	46	Y	
12/NW/0361	A study of Standard and New Antiepileptic Drugs – SANAD-II	14/03/13	30/04/13	47	Y	
10/H0802/13	Level of BP control and target organ damage in children with CKD - HOT KID	16/05/13	02/07/13	47	Y	
13/LO/1258	SMT C1100 - A Phase 1, Open-label, Single and Multiple Oral Dose, Safety, Tolerability and Pharmacokinetic Study in Paediatric Patients with Duchenne Muscular Dystrophy	07/11/13	24/12/2013	47	Y	
13/NW/0015	A pilot study of a mindfulness intervention designed for parents and carers of young people with Type 1 diabetes	14/02/13	04/04/13	49	Y	
12/NW/0766	PREDNOS 2	29/05/13	22/07/13	54	Y	

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70 day benchmark – Time to first patient recruitment

Performance in Initiating Clinical Research

Research Ethics Committee Reference Number	Name of Trial	Date of Receipt of Valid Research Application	Date of First Patient Recruitment	Duration Between VRA and First In	Benchmark Met	Comment
11/LO/0191	Night positioning on sleep deformity and pain in cerbral palsy	27/03/13	06/06/13	71	N	No patients seen within 70 days
13/YH/0201	THE EFFICACY, SAFETY AND TOLERABILITY OF SATIVEX AS AN ADJUNCTIVE TREATMENT TO EXISTING ANTISPASTICITY MEDICATIONS IN CHILDREN AGED 8 TO 18 YEARS WITH SPASTICITY DUE TO CEREBRAL PALSY OR TRAUMATIC CENTRAL NERVOUS SYSTEM INJURY WHO HAVE NOT RESPONDED ADEQUATELY TO THEIR EXISTING ANTI-SPASTICITY MEDICATIONS: A PARALLEL GROUP RANDOMISED, DOUBLE-BLIND, PLACEBOCONTROLLED STUDY FOLLOWED BY A 24-WEEK OPEN LABEL EXTENSION PHASE.	13/09/13	13/01/14	122	N	No patients seen within 70 days
13/LO/0010	MCRN204-Rituximab (A PHASE IIa, INTERNATIONAL, MULTICENTER, OPEN-LABEL, UNCONTROLLED STUDY TO EVALUATE THE SAFETY AND PHARMACOKINETICS OF 4 × 375 mg/m2 INTRAVENOUS RITUXIMAB IN PEDIATRIC PATIENTS WITH SEVERE GRANULOMATOSIS WITH POLYANGIITIS (WEGENER'S) OR MICROSCOPIC POLYANGIITIS)	21/03/13	18/09/13	181	N	No patients seen within 70 days and rare disorder
12/NW/0717	Fosaprepitant PK/PD CINV in Pediatric Cancer Patients	09/04/13	18/10/13	192	N	Issues with delayed Pharmacy set up and no patients seen
13/YH/0021	SD509 Mutsubishi Hyperphosphataemia CKD Colestilan (MCI-196-E16) (Multi-centre, Open-label Study Assessing the Efficacy, Safety and Tolerability of colestilan (MCI-196) in Paediatric Subjects with Chronic Kidney Disease (CKD) Stages 3b to 5 and Hyperphosphataemia (HP) not on Dialysis)	20/03/13	10/12/13	265	N	No patients seen within 70 days and rare disorder

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70 day benchmark – Time to first patient recruitment

Performance in Initiating Clinical Research

Research Ethics Committee Reference Number	Name of Trial	Date of Receipt of Valid Research Application	Date of First Patient Recruitment	Duration Between VRA and First In	Benchmark Met	Comment
13/NW/0321	A Phase Ib open label, multi-centre study to investigate the pharmacokinetics, pharmacodynamics, and safety of Tocilizumab following subcutaneous administration in patients with systemic juvenile idiopathic arthritis - WA28118	09/07/13			N	No patients seen within 70 days and rare disorder
12/LO/1628	Paediatric PK Ulcerative Colitis Study, version number: 6.0,04SEP2012	29/04/13			N	No patients seen within 70 days and rare disorder
13/YH/0020	SD529 Mitsubishi Dialysis CKD MCI-196-E14 (A Multi-centre, Randomized, Controlled, Parallel Group, Open-label Study Evaluating the Efficacy, Safety and Tolerability of Three Doses of Colestilan (MCI-196) Compared to Standard Therapy with a Calcium-based Phosphate Binder, in Paediatric Subjects with Chronic Kidney Disease Stage 5 on Dialysis and Hyperphosphataemia)	20/03/13			N	No patients seen within 70 days and rare disorder
13/YH/0022	SD530 Mitsubishi Ext CKD MCI-196-E15 (A Multi-centre, Flexible Dose, Parallel Group, Open-label, Active Control (Calcium-based Phosphate Binder), Long-term Extension Study Evaluating the Efficacy, Safety and Tolerability of Colestilan (MCI-196) in Paediatric Subjects With Hyperphosphataemia and With Either Chronic Kidney Disease Stage 5 on Dialysis or Chronic Kidney Disease Stages 3b to 5 Not on Dialysis)	20/03/13			N	No patients seen within 70 days and rare disorder

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70 day benchmark – Time to first patient recruitment

Analysis of Performance in Initiating Clinical Research

(70 day benchmark – Time to first patient recruitment)

- **Total Trials Reported – 25** *(Every clinical trial given NHS permission at Alder Hey within the previous 12 months (01/01/2013-31/12/2013)*
- **Total trials meeting the 70 day benchmark – 13** *(52% of reported trials) Clinical trials that have recruited the first participant within 70 days of a Valid Research Application*
- **Total trials still eligible to comply with 70 day benchmark – 3** *(12% of reported trials) Clinical trials where 70 day benchmark could still be met at end of reporting quarter*
- **Total trials NOT meeting the 70 day benchmark – 9** *(36% of reported trials) Clinical trials that either recruited the first patient after the 70 day target elapsed or have not yet recruited and 70 days have already elapsed*
- **Of trials not meeting 70 day benchmark (9), total trials where fault does not lie with NHS provider – 8** *(11% of reported trials) Clinical trials where reason for failure lies with NHS provider*

- **Mean number of Days between Valid Research Application and First Patient Recruited (# trials recruited to = 18) – 71 days**
- **Median number of Days between Valid Research Application and First Patient Recruited (# trials recruited to = 18) –47days**

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NIHR Central Commissioning Facility

Recruitment to time and target for commercial contract clinical trials

Performance in Delivery of Clinical Research

Research Ethics Committee Reference Number	Name of Trial	Target Number of Patients	Date Agreed to Recruit Target Number of Patients	Trial status	Target met within the agreed time	Comment
10/H1008/89	MCRN109 (FER-CKD-252) - A Randomized, Open-Label, Active-Controlled Study of the Safety, Efficacy, and Pharmacokinetics of Ferumoxytol Compared with Oral Iron for the Treatment of Iron Deficiency Anaemia in Paediatric Subjects with Nondialysis-dependent Chronic Kidney Disease	5	31/10/2013	Suspended	N/A	
10/H1008/105	MCRN110 - AMAG-FER-CKD-253	5	31/10/2013	Suspended	N/A	Suspended pending the unsuspension of MCRN109 lead in study.
12/NW/0694	A 12 week randomized, openlabel, active comparator period followed by a 12 week safety extension period to evaluate the safety and efficacy of Fesoterodine in subjects aged 6 to 16 years and >25 kg with symptoms of detrusor overactivity associated with a neurological condition (Neurogenic Detrusor Overactivity).	2	05/03/2014	Open	N/A	
13/NW/0276	European Survey of Paediatric Prolonged, Acute, Convulsive Seizures: Patient and Parent Experience of Current Practice in the Community Setting; Part of the PERFECT Initiative	10	13/03/2014	Open	N/A	
09/H0714/65	MCRN069 (CACZ885G2301E1) - An open-label extension study of canakinumab (ACZ885) in patients with Systemic Juvenile Idiopathic Arthritis (SJIA) and active systemic manifestations	3	01/06/2014	Open	N/A	
12/NW/0717	Fosaprepitant in Nausea & Vomiting	4	29/10/2014	Open	N/A	
13/LO/1258	SMT C1100 - A Phase 1, Open-label, Single and Multiple Oral Dose, Safety, Tolerability and Pharmacokinetic Study in Paediatric Patients with Duchenne Muscular Dystrophy	4	31/10/2014	Open	N/A	
13/LO/0010	MCRN204 (WA25615) - A phase IIa, international, multicenter, open-label, uncontrolled study to evaluate the safety and pharmacokinetics of 4 ? 375 mg/m2 intravenous rituximab in paediatric patients with severe granulomatosis with polyangiitis (Wegener's) or microscopic polyangiitis.	1	01/12/2014	Open	N/A	
10/H0718/64	MCRN095 (CACZ885D2307) - A one-year open-label, muticentre trial to assess efficacy, safety and tolerability of canakinumab (ACZ885) and the efficacy and safety of childhood vaccinations in patients aged 4 years or younger with Cryopyrin Associated Periodic Syndromes (CAPS)	1	23/12/2014	Open	N/A	
13/YH/0021	SD509 Mutsubishi Hyperphosphataemia CKD Colestilan (MCI-196-E16) (Multi-centre, Open-label Study Assessing the Efficacy, Safety and Tolerability of colestilan (MCI-196) in Paediatric Subjects with Chronic Kidney Disease (CKD) Stages 3b to 5 and Hyperphosphataemia (HP) not on Dialysis)	1	01/09/2015	Open	N/A	
13/YH/0020	SD529 Mitsubishi Dialysis CKD MCI-196-E14 (A Multi-centre, Randomized, Controlled, Parallel Group, Open-label Study Evaluating the Efficacy, Safety and Tolerability of Three Doses of Colestilan (MCI-196) Compared to Standard Therapy with a Calcium-based Phosphate Binder, in Paediatric Subjects with Chronic Kidney Disease Stage 5 on Dialysis and Hyperphosphataemia)	1	01/09/2015	Open	N/A	
12/YH/0020	MCRN170 (SPD503-318) - A Phase 3, Open-Label, Multicentre, Protocol To Provide Access To Guanfacine Hydrochloride Extended Release For European Subjects With Attention-Deficit/Hyperactivity Disorder (ADHD) Who Participated In Study SPD503-315 Or SPD503-316	1	01/11/2015	Open	N/A	

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Recruitment to time and target for commercial contract clinical trials

Performance in Delivery of Clinical Research

Research Ethics Committee Reference Number	Name of Trial	Target Number of Patients	Date Agreed to Recruit Target Number of Patients	Trial status	Target met within the agreed time	Comment
13/NW/0321	A Phase Ib open label, multi-centre study to investigate the pharmacokinetics, pharmacodynamics, and safety of Tocilizumab following subcutaneous administration in patients with systemic juvenile idiopathic arthritis - WA28118	1	01/12/2015	Open	N/A	
13/NW/0320	A Phase Ib open label, multi-centre study to investigate the pharmacokinetics, pharmacodynamics, and safety of Tocilizumab following subcutaneous administration in patients with Polyarticular-Course Juvenile Idiopathic Arthritis - WA28117	1	01/12/2015	Open	N/A	
11/SC/0454	MCRN214 (WA28029) - A STUDY TO EVALUATE DECREASED DOSE FREQUENCY IN PATIENTS WITH ACTIVE SYSTEMIC JUVENILE IDIOPATHIC ARTHRITIS (SJIA) WHO EXPERIENCE LABORATORY ABNORMALITIES DURING TREATMENT WITH TOCILIZUMAB.	2	17/12/2015	Open	N/A	
13/YH/0201	THE EFFICACY, SAFETY AND TOLERABILITY OF SATIVEX AS AN ADJUNCTIVE TREATMENT TO EXISTING ANTISPASTICITY MEDICATIONS IN CHILDREN AGED 8 TO 18 YEARS WITH SPASTICITY DUE TO CEREBRAL PALSY OR TRAUMATIC CENTRAL NERVOUS SYSTEM INJURY WHO HAVE NOT RESPONDED ADEQUATELY TO THEIR EXISTING ANTI-SPASTICITY MEDICATIONS: A PARALLEL GROUP RANDOMISED, DOUBLE-BLIND, PLACEBOCONTROLLED STUDY FOLLOWED BY A 24-WEEK OPEN LABEL EXTENSION PHASE.	8	30/12/2015	Open	N/A	
13/YH/0022	SD530 Mitsubishi Ext CKD MCI-196-E15 (A Multi-centre, Flexible Dose, Parallel Group, Open-label, Active Control (Calcium-based Phosphate Binder), Long-term Extension Study Evaluating the Efficacy, Safety and Tolerability of Colestilan (MCI-196) in Paediatric Subjects With Hyperphosphataemia and With Either Chronic Kidney Disease Stage 5 on Dialysis or Chronic Kidney Disease Stages 3b to 5 Not on Dialysis)	1	31/03/2016	Open	N/A	
07/H0904/84	The national programme for enhanced pneumococcal surveillance - The national programme for enhanced pneumococcal surveillance of complicated pneumococcal pneumonia and empyema in UK children	60	01/08/2016	Open	N/A	
11/LO/0923	NCRN259 HERBY - Bevacizumab in paediatric high grade glioma - A phase II open-label, randomized, multi-centre comparative study of bevacizumab-based therapy in paediatric patients with newly diagnosed supratentorial high-grade glioma	2	22/07/2017	Open	N/A	
08/H0718/11	BERNIE	7	01/08/2017	Open	N/A	
11/YH/0010	MILLENIUM	16	31/12/2017	Open	N/A	
11/EM/0014	MCRN126 (F506-CL-0404) - A Long-term, Open-label, Non-comparative Study to Evaluate theSafety and Efficacy of a Modigraf® Based Immunosuppression Regimen in Paediatric Solid Allograft Recipients.	2	01/03/2018	Open	N/A	
12/NW/0367	MCRN177 (BEL114055) - A multi-centre, randomised, parallel group, placebo-controlled double-blind trial to evaluate the safety, efficacy and pharmacokinetics of belimumab, a human monoclonal anti-BLyS antibody, plus standard therapy in paediatric patients with Systemic Lupus Erythematosus (SLE)	2	13/04/2026	Open	N/A	

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Recruitment to time and target for commercial contract clinical trials

Performance in Delivery of Clinical Research

Research Ethics Committee Reference Number	Name of Trial	Target Number of Patients	Date Agreed to Recruit Target Number of Patients	Trial status	Target met within the agreed time	Comment
MCRN148	MCRN148 (N01357) - Observational sentinel sites study (N01357) in infants younger than 12 months who are prescribed the treatment with KEPPRA® (Levetiracetam) oral solution in usual clinical practice.	3	30/11/2013	Closed - In Follow Up	Y	
13/LO/0566	A Phase 3, 2-Part, Open-Label Study to Evaluate the Safety, Pharmacokinetics, and Pharmacodynamics of Ivacaftor in Subjects With Cystic Fibrosis Who are 2 Through 5 Years of Age and Have a CFTR Gating Mutation	1	30/09/2013	Closed - In Follow Up	Y	
08/H0504/179	MCRN035 (BUP1501) - A multi-centre, open-label, single therapy, dose ranging study to characterise the pharmacokinetics and tolerability of BTDS 5-20 µg/h in children who require opioid analgesia for moderate to severe mouth pain secondary to chemotherapy induced mucositis	3	31/01/2013	Closed - In Follow Up	Y	
10/H1306/78	MCRN112 (SPD503-315) - A phase 3 double blind placebo controlled multi centre randomised withdrawal long term maintenance of efficacy and safety study of extended release Guanfacine Hydrochloride in Children and Adolescents aged 6-17 with Attention Deficit/ Hyperactivity Disorder	2	03/09/2012	Closed - In Follow Up	Y	
MCRN049	MCRN049 (WA19977A) - A multi-center international study to evaluate the efficacy and safety of tocilizumab in subjects with active polyarticular-course juvenile idiopathic arthritis; followed by an open-label extension to examine the long term use of tocilizumab.	4	01/03/2011	Closed - In Follow Up	Y	
MCRN036	MCRN036 (PTC124-GD-009-CF) - A Phase 3 Efficacy and Safety Study of PTC124 as an Oral Treatment for Nonsense-Mutation-Mediated Cystic Fibrosis	1	21/02/2012	Closed - In Follow Up	Y	
MCRN067	MCRN067 (CACZ885G2301) - A randomized, double-blind, placebo controlled, withdrawal study of flare prevention of canakinumab (ACZ885) in patients with Systemic Juvenile Idiopathic Arthritis (SJIA) and active systemic manifestations	2	01/08/2010	Closed - Follow Up Complete	Y	
MCRN011	MCRN011 (WA18221) - A 12-week randomized, double blind, placebo-controlled, parallel group, 2-arm study to evaluate the efficacy and safety of tocilizumab in patients with active systemic juvenile idiopathic arthritis (sJIA); with a 92-week single arm open-label extension to examine the long term use of tocilizumab	2	30/06/2011	Closed - Follow Up Complete	Y	
MCRN068	MCRN068 (CACZ885G2305) - A randomized, double-blind, placebo controlled, single-dose study to assess the initial efficacy of canakinumab (ACZ885) with respect to the adapted ACR Pediatric 30 criteria in patients with Systemic Juvenile Idiopathic Arthritis (SJIA) and active systemic manifestations	2	15/07/2011	Closed - Follow Up Complete	Y	
12/LO/1628	MCRN187 (SPD476-112) - A Phase 1, Multicenter, Open-label Study to Determine the Safety and Pharmacokinetics of MMX Mesalamine Following Administration in Children and Adolescents With Ulcerative Colitis	1	26/06/2013	Closed - In Follow Up	N	Global study target met early and study closed by Sponsor

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Reporting Period Q3 13/14 – 01/01/2013 to 31/12/2013

NIHR Central Commissioning Facility

Recruitment to time and target for commercial contract clinical trials

Alder Hey Children's NHS
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Analysis of Performance in Delivery of Clinical Research

(Recruitment to Time and Target)

- **Total Trials Reported – 33** *(Clinical trials hosted by Alder Hey Children's NHS FT within a 12 month period (01/01/2013-31/12/2013)*
- **Total Trials Open – 23*** *(70% of reported trials) Clinical trials open to recruitment and have therefore not yet reached their end date or target*
- **Total Trials Closed – 10** *(30% of reported trials) Clinical trials now closed to recruitment and in Follow Up or Follow Up Complete status*
- **Total Trials Closed NOT Meeting Time and Target – 1** *Reasons: 1) Global study recruitment target met early and study closed by sponsor*
- **Total Trials Closed Meeting Time and Target – 9** *(90% of closed trials)*

** Includes two trials currently in suspend*