These Clinical Study Results are provided for informational purposes only.

This lay summary is a brief summary of the main results from a clinical study. The study listed may include approved and non-approved uses, formulations or treatment regimens. It is not intended to promote any product or indication and is not intended to replace the advice of a health care professional. The results reported in any single clinical trial may not reflect the results obtained across the full clinical development program. Only a physician can determine if a specific product is the appropriate treatment for a particular patient. If you have questions, please consult a health care professional. Before prescribing any product, healthcare professionals should consult the regional approved product labeling for indications and proper use of the product.

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Clinical Study Results

Efficacy and Safety of Burosumab (KRN23) Versus Oral Phosphate and Active Vitamin D Treatment in Pediatric Patients with X-Linked Hypophosphatemia (XLH)

Thank you!

Participants in clinical studies belong to a large community of people who take part in clinical research around the world. Thank you to the children, parents, and caregivers who took part in the clinical study for burosumab, also called KRN23. Ultragenyx and Kyowa Kirin, the sponsors of this study, are grateful to those who participated and believe it is important to share the results.

What does this summary cover?

This study was done to compare burosumab against standard of care (a combination of oral phosphate and active vitamin D) for X-Linked Hypophosphatemia (XLH).

This summary shows the main results from the UX023-CL301 study and was created to help people learn:

- What XLH is
- Why this study was done
- Who was allowed to participate in this study
- What medicines the participants of this study received
- What results were measured in this study
- What side effects were reported during this study
- How this study helped people with XLH and the healthcare community
- Where to find more information about this study

It may be helpful to review this document with your doctor or healthcare provider.

Protocol Number

UX023-CL301

NCT Number

NCT02915705

Treatments Studied

- Burosumab
- Oral Phosphate and Vitamin D

Study Sponsors

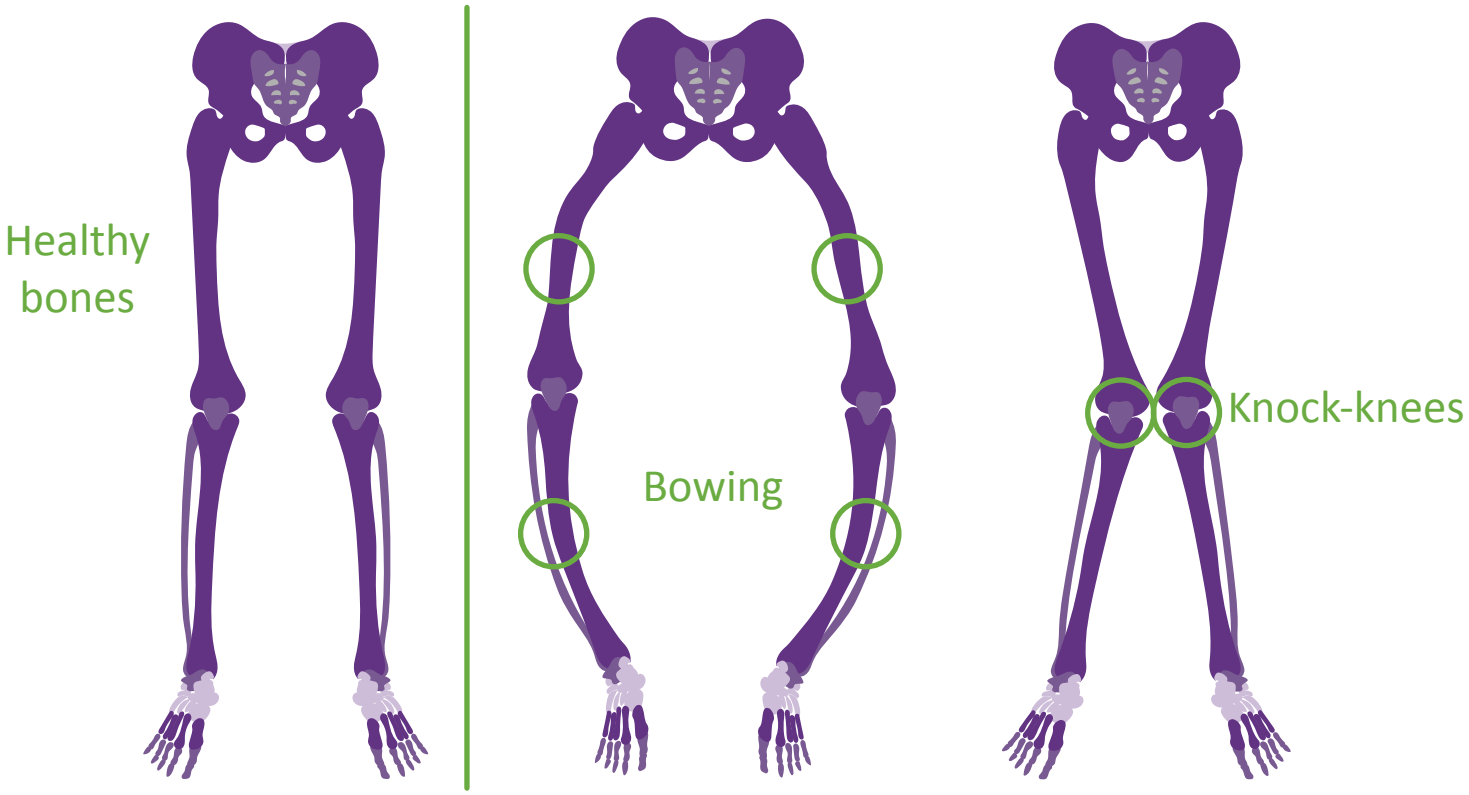
Ultragenyx and Kyowa Kirin

Study Dates

September 2016 to July 2019

What is XLH?

XLH is a rare and life-long bone disease that affects the lives of children and adults. XLH is a disease where the body loses too much phosphate. Phosphate is needed to help create healthy bones. In children these low levels of phosphate can cause a bone condition called rickets, where the bones become soft and weak and can cause the bones of the legs to bend away from each other (bowing) or bend towards each other (knock-knees).



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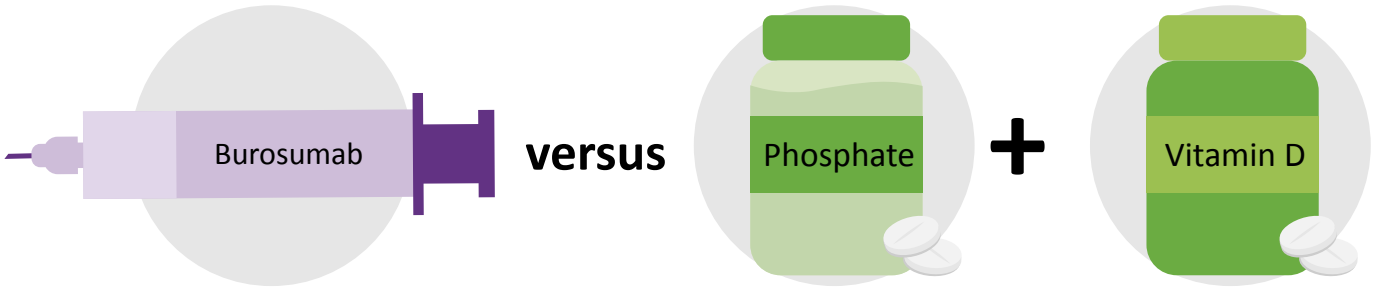
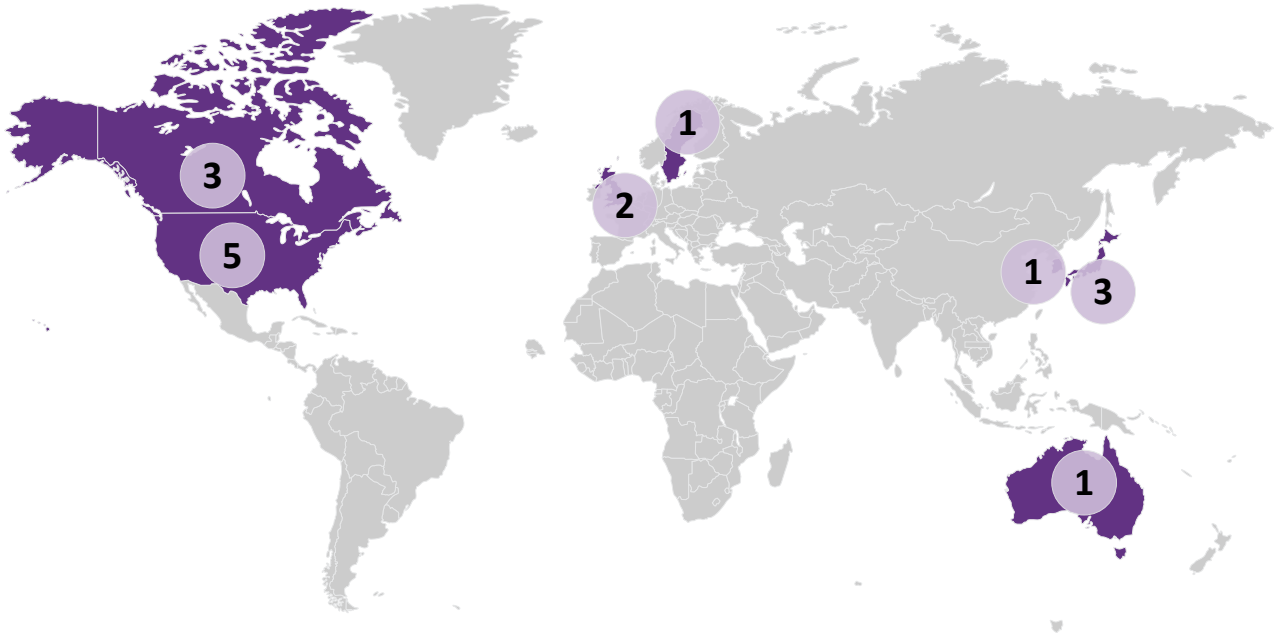


In this study, the researchers wanted to see how well burosumab worked when compared to standard of care (a combination of oral phosphate and active vitamin D). The researchers also wanted to find out if the children had any side effects during the study. Side effects are unwanted or unexpected experiences that children may have that the doctors think might be related to the study treatment.

Where was this study done?

This study took place at 16 clinical sites: 5 in the USA, 3 in Japan, 3 in Canada, 2 in the UK, 1 in Sweden, 1 in South Korea, and 1 in Australia.



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Children with XLH were allowed to participate in this study if they met specific criteria. All the criteria can be found here: https://clinicaltrials.gov/ct2/show/NCT02915705.

Below are some key criteria for who could participate in the study:

Boys or girls, aged 1 to 12 years, with rickets, as determined by x-rays

Specific low levels of phosphate in the blood

Laboratory test results showing that children had XLH

Received standard of care supplements for a certain amount of time (based on the child's age)

What medicines did the children in this study receive?

61

children with XLH took part in this study

Before the start of the study, all children had to stop standard of care supplements for **7 days**. This step was taken to ensure no amount of standard medicine remained in the children's bodies at the start of the study.

This was an open-label study. This means that everyone knew what medicine they were taking but neither the child nor the study doctor could choose the treatment they received. Children were put into one of two treatment groups by chance (randomized). This is like flipping a coin or drawing numbers out of a hat.

Burosumab Group

0.8 mg/kg starting dose

Given every 2 weeks as an injection under the skin

There were:
29 children
(16 girls, 13 boys)
in this group

Standard of Care Group

Multiple doses per day of phosphate and one or more daily doses of active vitamin D

Given daily by mouth

There were:
32 children
(18 girls, 14 boys)
in this group

40 weeks

Results were collected.

64 weeks

Results were collected.

All children in Japan and Korea stopped the study at week 64

At week 64 the study was extended and children taking standard of care supplements switched to receive burosumab

Children already in the burosumab group continued to receive burosumab

140 weeks

Results were collected.

Understanding the study results.

This is a result summary of the main question the researchers wanted to answer, and other results that researchers believe may be interesting to the children and their caregivers. Individual results of each child might be different and are not in this summary. If you or a child in your care who participated in this study have questions about individual results, please contact the doctor or staff at your study site.

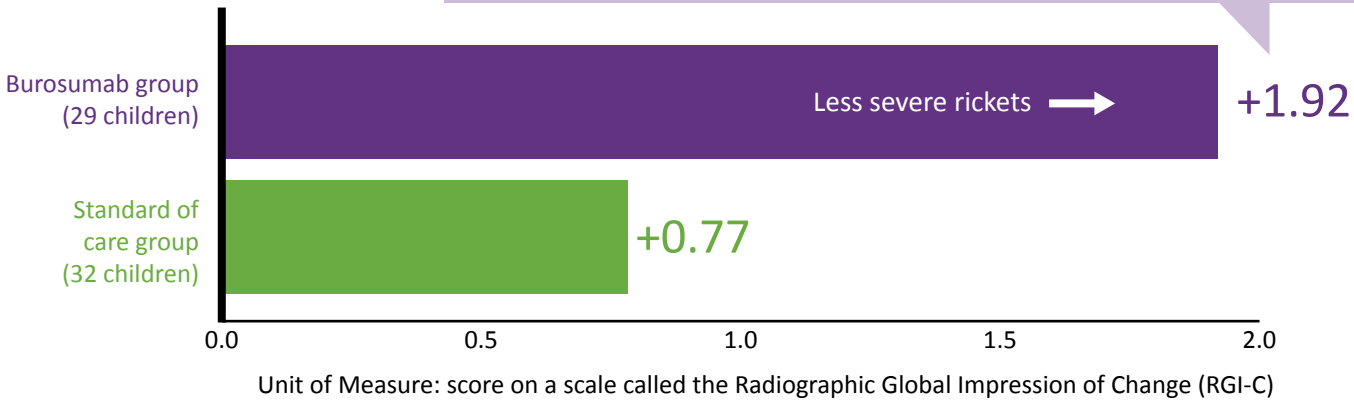
What was the main result measured in this study?

Measuring change in rickets at week 40

An important treatment goal in children with XLH is to heal rickets, a condition where the bones become soft and weak. To measure changes in rickets, researchers compared x-rays of the children's wrists and knees before and after starting treatment (burosumab or standard of care) to determine the Radiographic Global Impression of Change (RGI-C) score. This score measures if there was healing, worsening or no change with treatment.

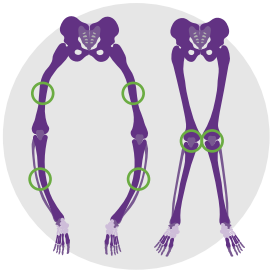
How did the study medications affect a child's rickets at week 40?

Rickets severity decreased more in children who received burosumab compared to children treated with standard of care at week 40.



Unit of Measure: score on a scale called the Radiographic Global Impression of Change (RGI-C)

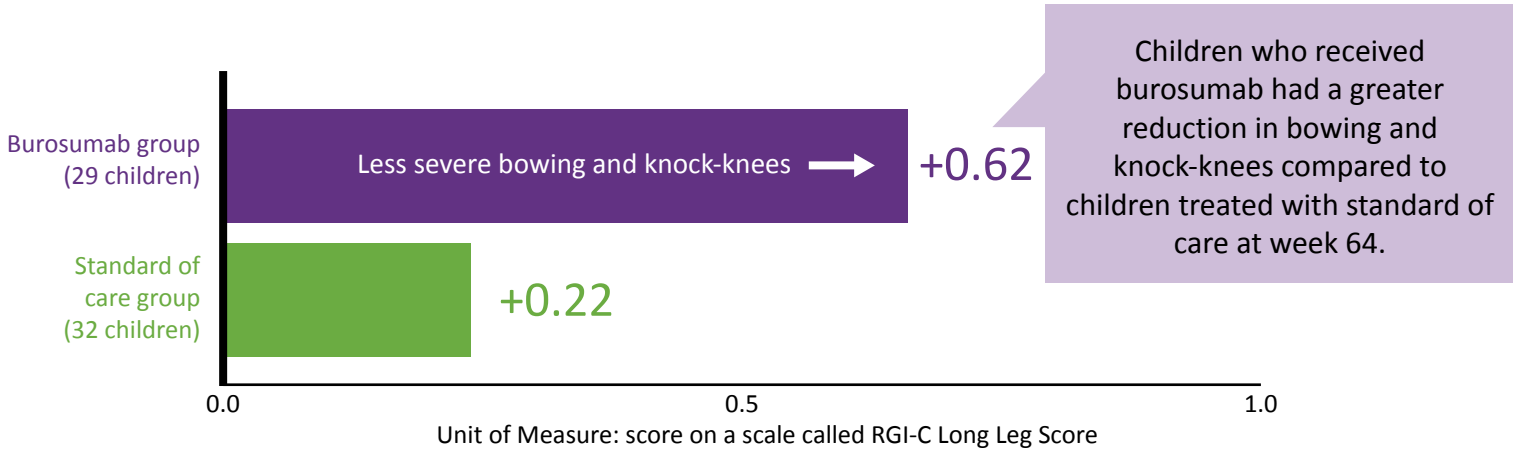
What were some other results measured in this study?

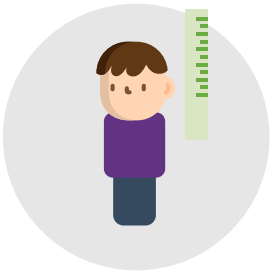


Measuring bowing and knock-knees at week 64

The RGI-C score from the x-rays of legs of children was also used to measure the severity of leg bowing and knock-knees.

How did the study medications affect the shape of the legs at week 64?

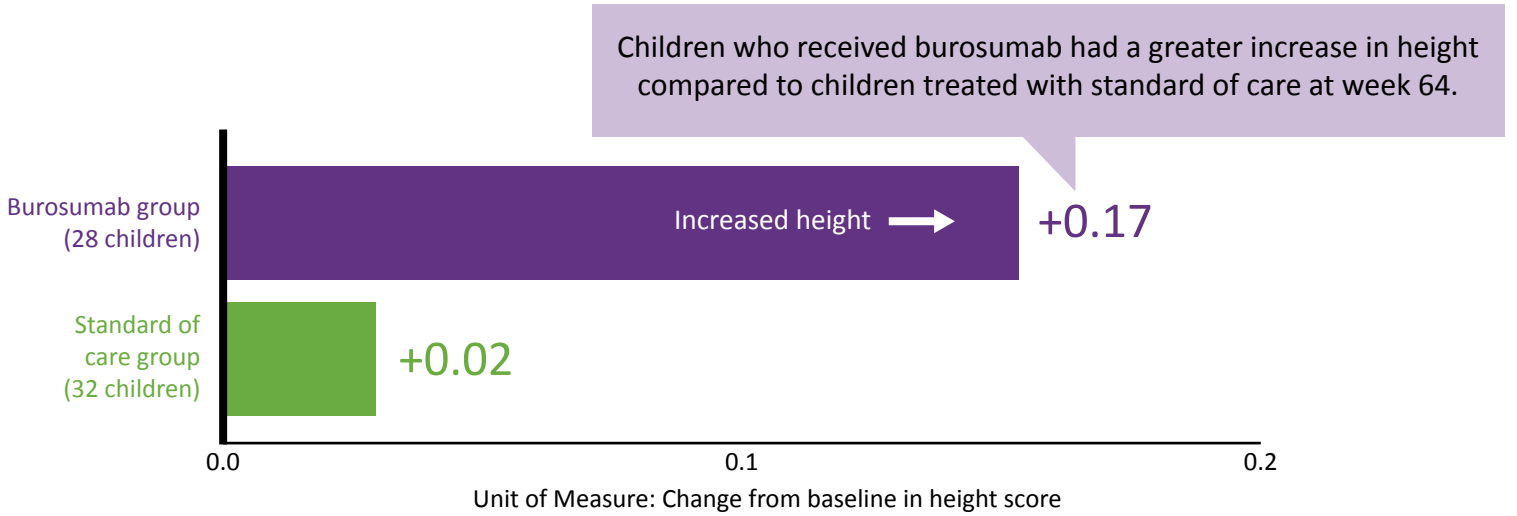




Measuring the height of children at week 64

Height was measured and compared to the height of children of the same age and sex, who did not have XLH. This provided a height score.

How did the study medications affect a change in height at week 64?



What were some other results measured in this study?

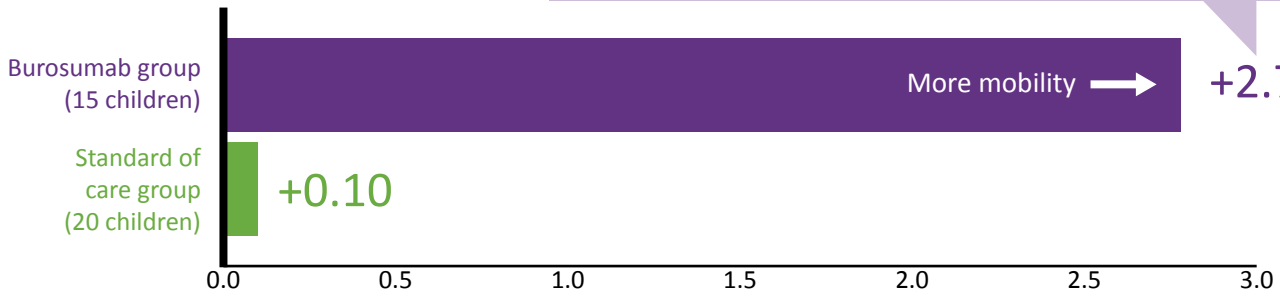
Measuring the general well-being of a child at week 40

Questions from a tool called PROMIS (Patient-Reported Outcomes Measurement Information System) were used in children 5 years or older to measure their well-being in terms of mobility, tiredness and how much pain interfered with activities.

How did the study medications affect children's well-being?

A greater increase in mobility was reported for children who received burosumab in comparison to children treated with standard of care at week 40.

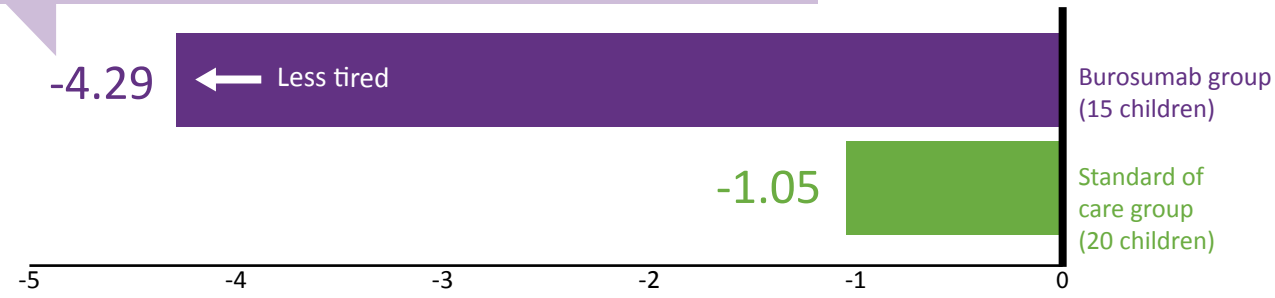
Measurement of Mobility



Unit of Measure: Change from baseline in score on a scale called PROMIS specifically for mobility

A greater reduction in being tired was reported for children who received burosumab in comparison to children treated with standard of care at week 40.

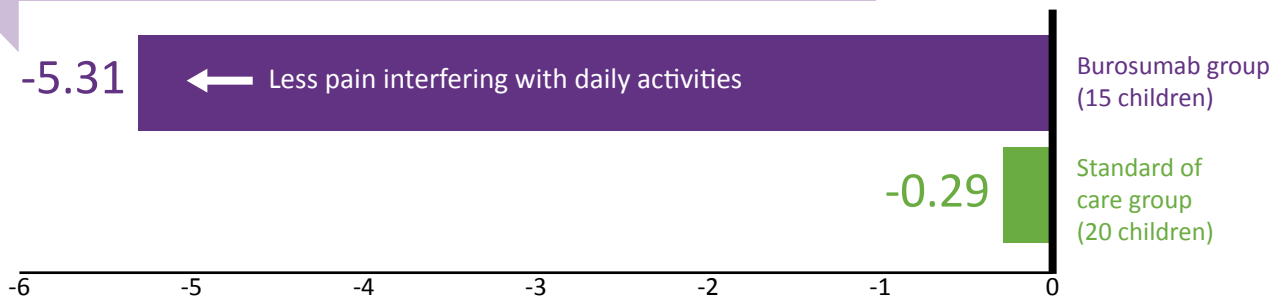
Measurement of Tiredness



Unit of Measure: Change from baseline in score on a scale called PROMIS specifically for tiredness

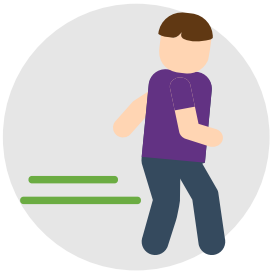
A greater reduction in pain interference was reported for children who received burosumab in comparison to children treated with standard of care at week 40.

Measurement of Pain Interference



Unit of Measure: Change from baseline in score on a scale called PROMIS specifically for pain

What were some other results measured in this study?

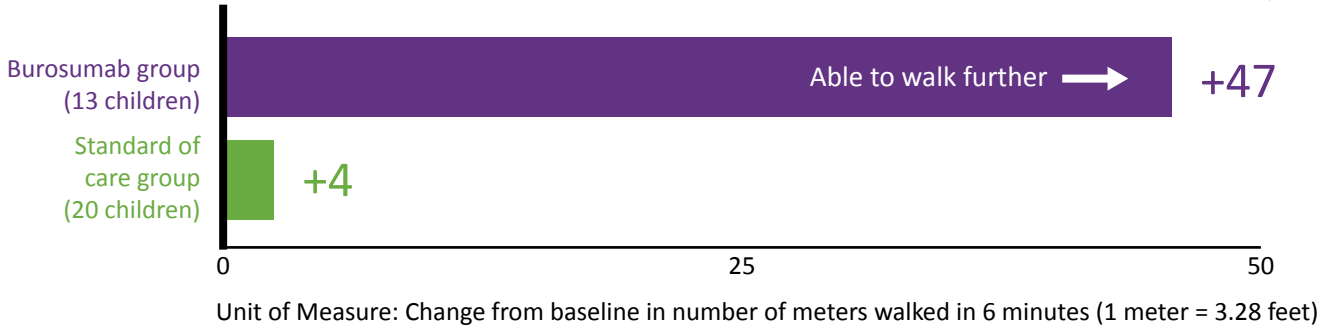


Measuring how far a child could walk at week 40

This was measured in children ages 5 or older. Researchers measured the distance each child could walk in 6 minutes.

How did the study medications affect how far a child could walk in 6 minutes at week 40?

Children who received burosumab showed a greater increase in how far they could walk in 6 minutes compared to children who received standard of care at week 40.



Unit of Measure: Change from baseline in number of meters walked in 6 minutes (1 meter = 3.28 feet)

What side effects were reported up to week 64 of this study?

Side effects may or may not be related to the medicines given in the study. Side effects may also be related to other medicines taken while in the study, to the child's other medical conditions, or to any tests performed in the study.

This section is a summary of the side effects that happened during this study that the doctors thought might be related to the study medicine taken.

A side effect is considered “serious” when it causes death or is life-threatening, causes a birth defect or disability, or requires hospital care.

What side effects were reported up to week 64 of this study?

Side effects possibly related to treatment		Number of children reported to have side effects
 How many children had side effects? (all side effects reported by children including those thought to be serious)	Burosumab	**17 children** out of 29 (58.6%)
	Standard of care	**8 children** out of 32 (25.0%)
 How many children had serious side effects?	Burosumab	**0 children** out of 29 (0%)
	Standard of care	**0 children** out of 32 (0%)

No child died during this study or stopped taking the study treatment because of side effects or serious side effects.

What side effects were reported up to week 64 of this study?

The graphic below shows the side effects reported up to week 64 that:

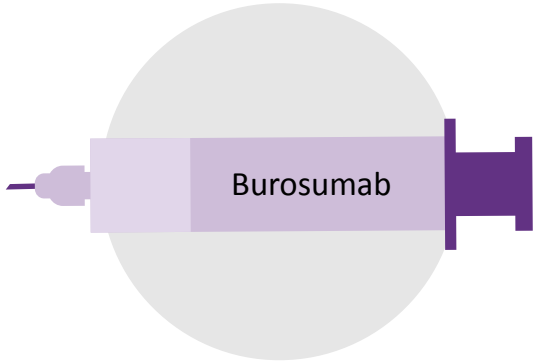
- doctors thought might be related to study drug
- happened in more than 10% of all the children in this study
- there were other side effects, but these happened in fewer children

Side effects possibly related to treatment	Number of children reported to have side effects
	Burosumab = child affected = child not affected **Standard of care** = child affected = child not affected
 Reddening of the skin at the injection site	**8 children** out of 29 (27.6%) **0 children** out of 32 (0%)
 Pain in arms, hands, legs or feet	**7 children** out of 29 (24.1%) **2 children** out of 32 (6.3%)
 Skin reaction at the injection site	**7 children** out of 29 (24.1%) **0 children** out of 32 (0%)
 Pain in joints	**6 children** out of 29 (20.7%) **4 children** out of 32 (12.5%)
 Infection in tooth	**4 children** out of 29 (13.8%) **0 children** out of 32 (0%)
 Itching at the injection site	**3 children** out of 29 (10.3%) **0 children** out of 32 (0%)
 Rash at the injection site	**3 children** out of 29 (10.3%) **0 children** out of 32 (0%)
 Swelling at the injection site	**3 children** out of 29 (10.3%) **0 children** out of 32 (0%)

When reading this information, it is important to remember that the standard of care was given by mouth, and not an injection.

After Week 64, children who took standard of care switched to receive burosumab for up to week 140. Children who had been taking burosumab continued receiving burosumab for up to week 140. Overall, the side effects were the same as during the first 64 weeks.

How has this study helped people with XLH and researchers?



Overall, the researchers found that burosumab was more effective than standard of care in reducing the severity of rickets, bowing or knock-knees, and tiredness and increased growth and the ability to walk further, in children with XLH.

There were more side effects considered related to burosumab than standard of care supplements. These side effects were most often related to injecting burosumab under the skin.

The results presented here are for one study. Other studies may provide new information or different results. Always talk to a doctor before making any treatment changes.

Further clinical research with burosumab is ongoing.

Where can I find more information about this study?

You can find more information about this study including a report of the study's results on these websites:

- https://clinicaltrials.gov/ct2/show/NCT02915705
- https://www.clinicaltrialsregister.eu/ctr/ Search: 2016-000600-29

If you have questions about the results, please speak with your doctor or staff at the study site.

The phone number for Ultragenyx is: 415-483-8800 and the email is: patientadvocacy@ultragenyx.com

Thank you

At Ultragenyx, our focus is developing medicines for people who live with rare and ultra-rare diseases. But it takes more than scientific knowledge and research to develop medications. Your involvement is essential and ensures that the research process moves forward. Thank you for your participation in this study and commitment to research.

ultragenyx pharmaceutical

Ultragenyx is a biopharmaceutical company committed to bringing products to patients for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases.

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Kyowa KIRIN

Kyowa Kirin commits to innovate drug discovery driven by state-of-the-art technologies. The company focuses on creating new values in the four therapeutic areas: nephrology, oncology, immunology/allergy and neurology.

www.kyowakirin.com

Clinical Study Results

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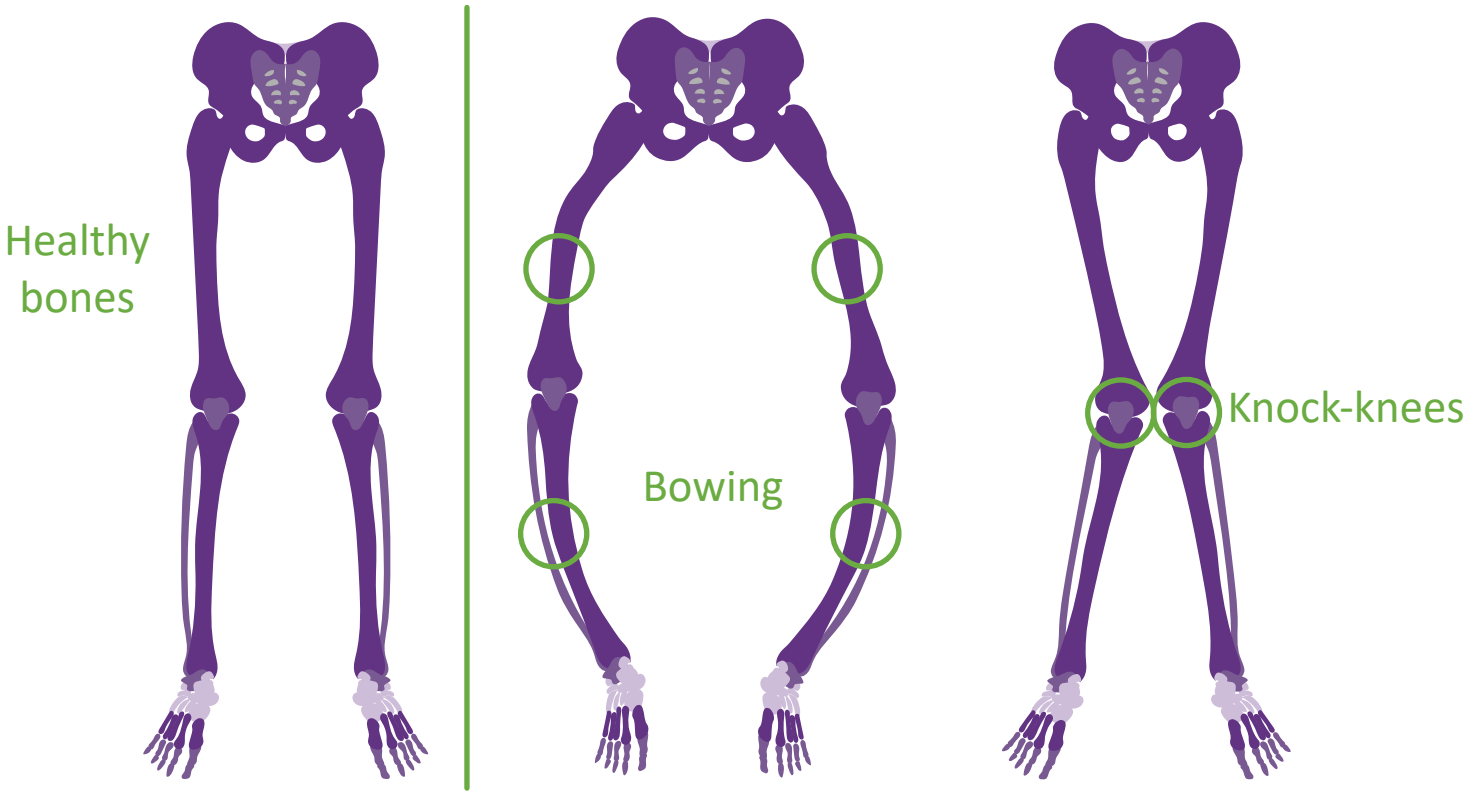
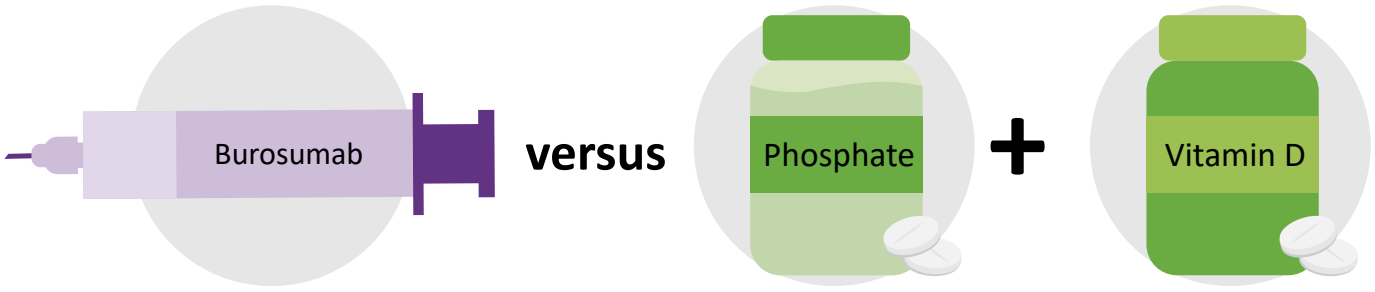


Why was this study done?



In this study, the researchers wanted to see how well burosumab worked when compared to standard of care (a combination of oral phosphate and active vitamin D). The researchers also wanted to find out if the children had any side effects during the study. Side effects are unwanted or unexpected experiences that children may have that the doctors think might be related to the study treatment.

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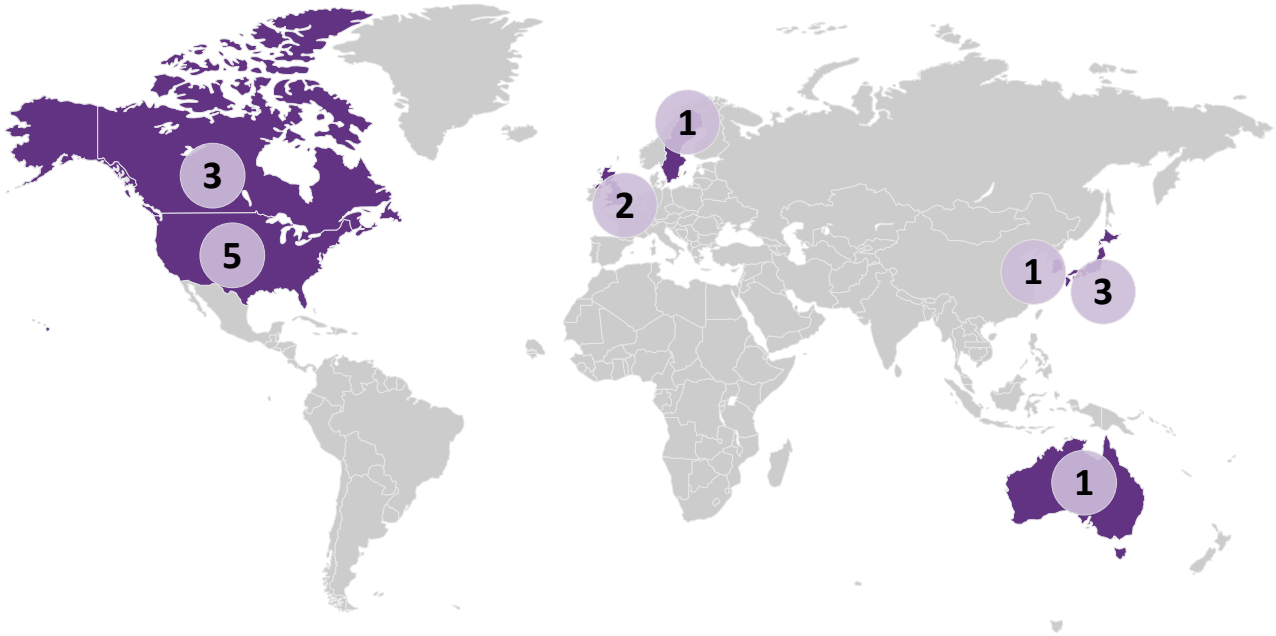

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What was the main result measured in this study?

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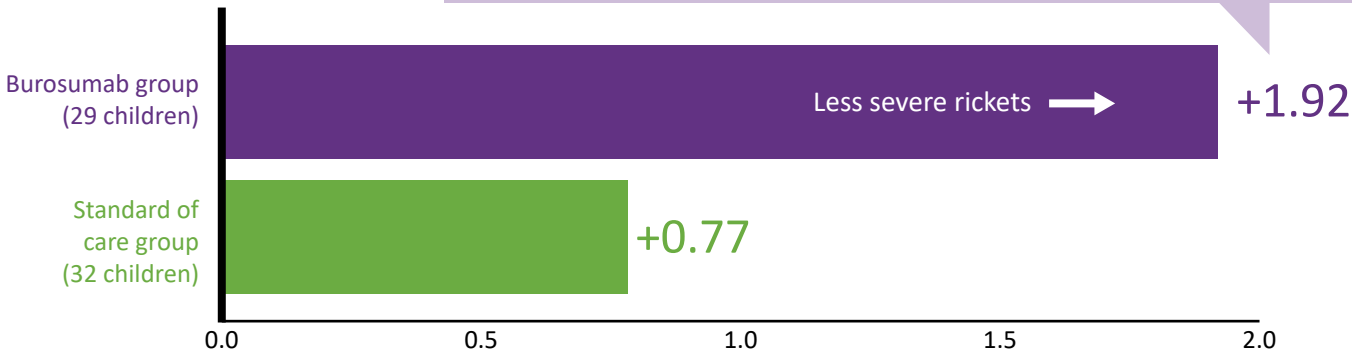
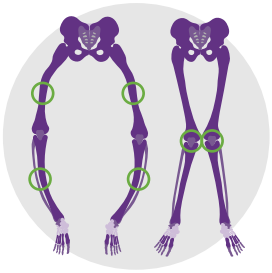
How did the study medication affect a child's rickets at week 40?

Rickets severity decreased more in children who received burosumab compared to children treated with standard of care at week 40.



Unit of Measure: score on a scale called the Radiographic Global Impression of Change (RGI-C)

What were some other results measured in this study?



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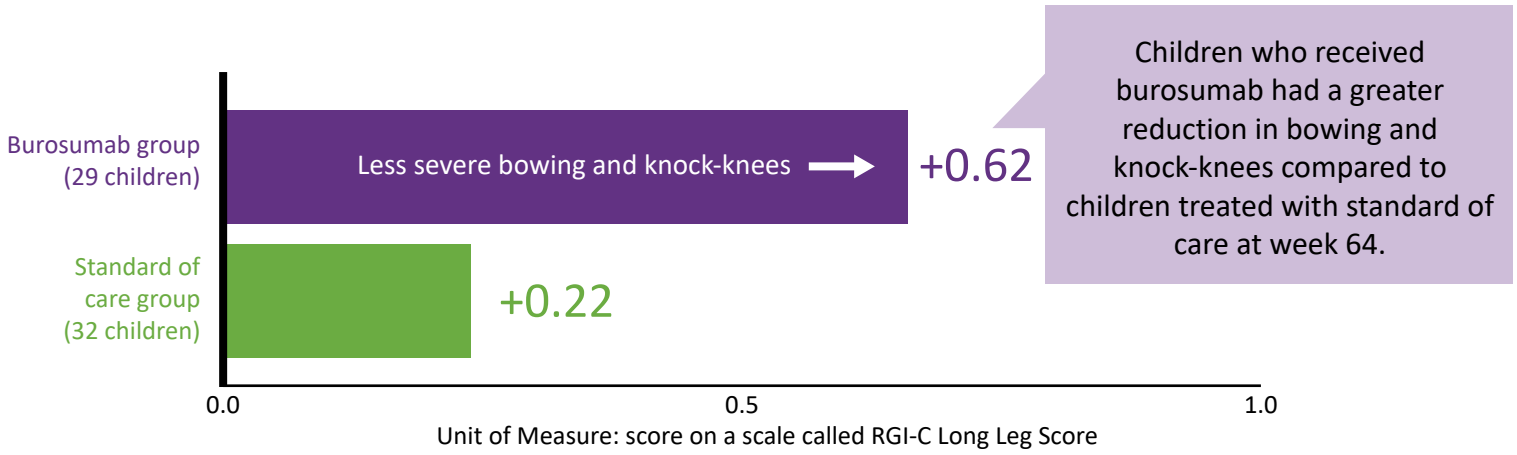
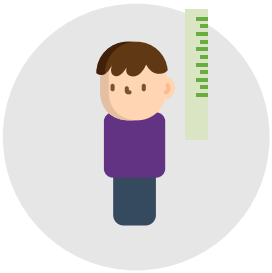
How did the study medication affect the shape of the legs at week 64?





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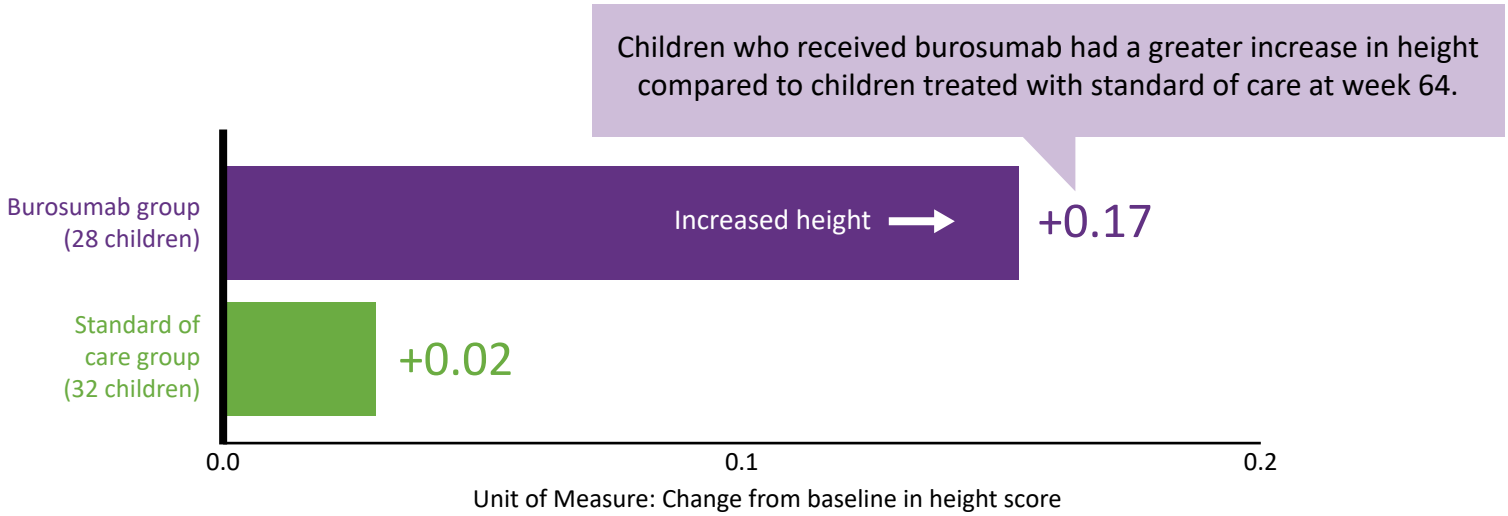

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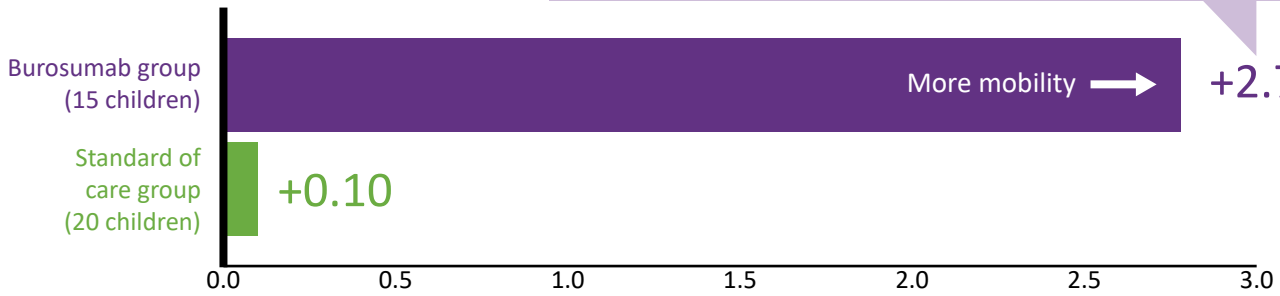
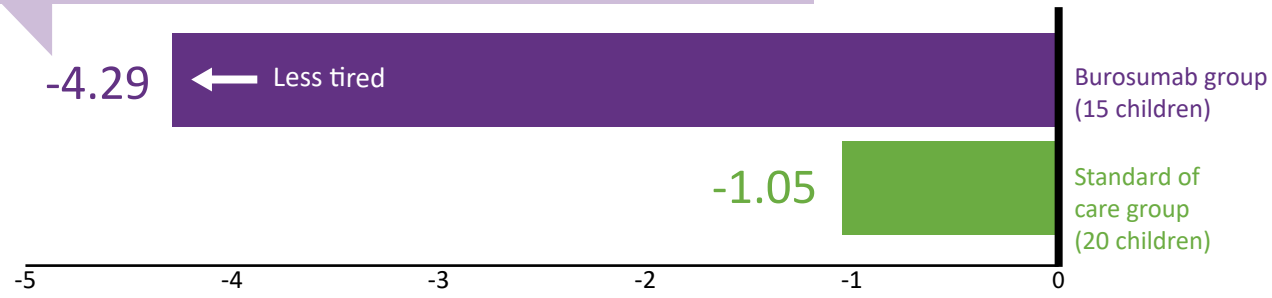
Measurement of Mobility



Unit of Measure: Change from baseline in score on a scale called PROMIS specifically for mobility

A greater reduction in being tired was reported for children who received burosumab in comparison to children treated with standard of care at week 40.

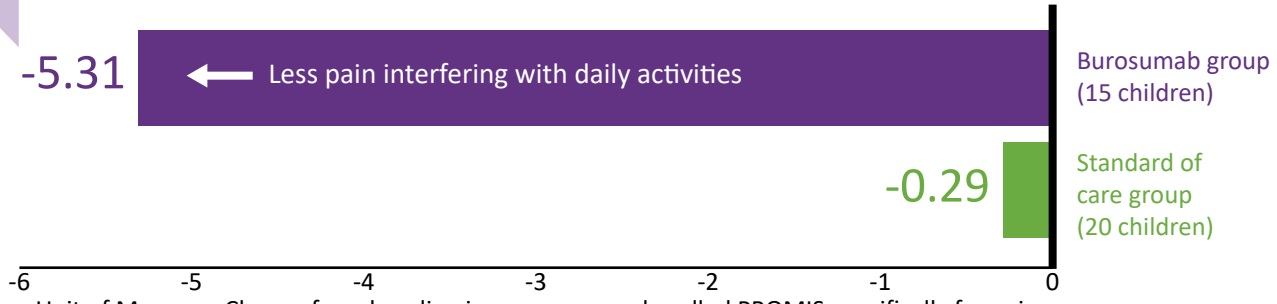
Measurement of Tiredness



Unit of Measure: Change from baseline in score on a scale called PROMIS specifically for tiredness

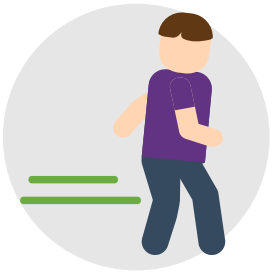
A greater reduction in pain interference was reported for children who received burosumab in comparison to children treated with standard of care at week 40.

Measurement of Pain Interference



Unit of Measure: Change from baseline in score on a scale called PROMIS specifically for pain

What were some other results measured in this study?

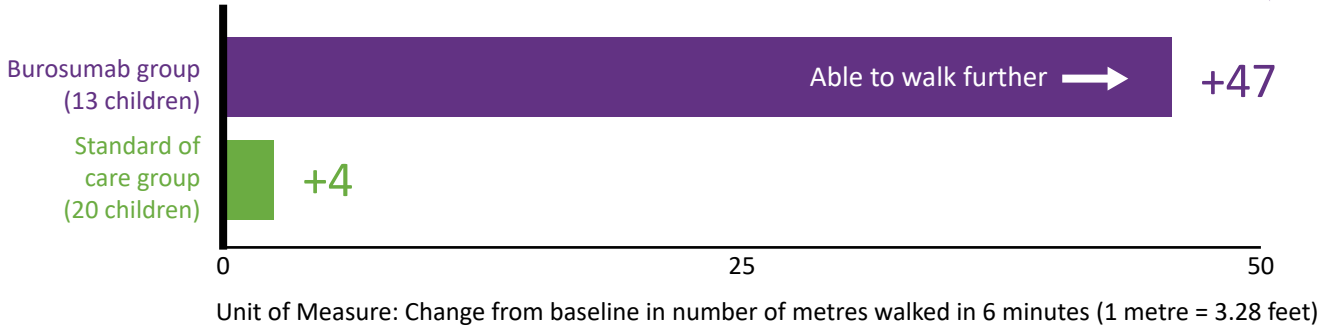


Measuring how far a child could walk at week 40

This was measured in children ages 5 or older. Researchers measured the distance each child could walk in 6 minutes.

How did the study medication affect how far a child could walk in 6 minutes at week 40?

Children who received burosumab showed a greater increase in how far they could walk in 6 minutes compared to children who received standard of care at week 40.



Unit of Measure: Change from baseline in number of metres walked in 6 minutes (1 metre = 3.28 feet)

What side effects were reported up to week 64 of this study?

Side effects may or may not be related to the medicines given in the study. Side effects may also be related to other medicines taken while in the study, to the child's other medical conditions, or to any tests performed in the study.

This section is a summary of the side effects that happened during this study that the doctors thought might be related to the study medicine taken.

A side effect is considered “serious” when it causes death or is life-threatening, causes a birth defect or disability, or requires hospital care.

What side effects were reported up to week 64 of this study?

Side effects possibly related to treatment		Number of children reported to have side effects
How many children had side effects? (all side effects reported by children including those thought to be serious)	Burosumab	**17 children** out of 29 (58.6%)
	Standard of care	**8 children** out of 32 (25.0%)
How many children had serious side effects?	Burosumab	**0 children** out of 29 (0%)
	Standard of care	**0 children** out of 32 (0%)

No child died during this study or stopped taking the study treatment because of side effects or serious side effects.

What side effects were reported up to week 64 of this study?

The graphic below shows the side effects reported up to week 64 that:

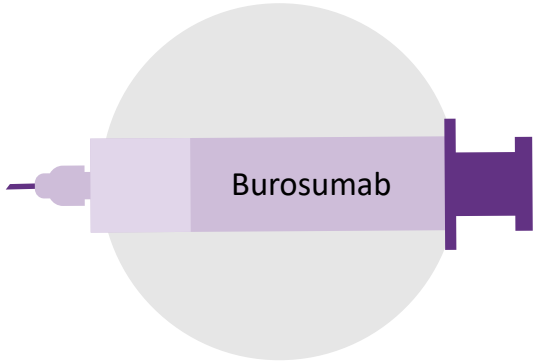
- doctors thought might be related to study drug
- happened in more than 10% of all the children in this study
- there were other side effects, but these happened in fewer children

Side effects possibly related to treatment	Number of children reported to have side effects
	Burosumab = child affected = child not affected **Standard of care** = child affected = child not affected
 Reddening of the skin at the injection site	**8 children** out of 29 (27.6%) **0 children** out of 32 (0%)
 Pain in arms, hands, legs or feet	**7 children** out of 29 (24.1%) **2 children** out of 32 (6.3%)
 Skin reaction at the injection site	**7 children** out of 29 (24.1%) **0 children** out of 32 (0%)
 Pain in joints	**6 children** out of 29 (20.7%) **4 children** out of 32 (12.5%)
 Infection in tooth	**4 children** out of 29 (13.8%) **0 children** out of 32 (0%)
 Itching at the injection site	**3 children** out of 29 (10.3%) **0 children** out of 32 (0%)
 Rash at the injection site	**3 children** out of 29 (10.3%) **0 children** out of 32 (0%)
 Swelling at the injection site	**3 children** out of 29 (10.3%) **0 children** out of 32 (0%)

When reading this information, it is important to remember that the standard of care was given by mouth, and not an injection.

After Week 64, children who took standard of care switched to receive burosumab for up to week 140. Children who had been taking burosumab continued receiving burosumab for up to week 140. Overall, the side effects were the same as during the first 64 weeks.

How has this study helped people with XLH and researchers?



Overall, the researchers found that burosumab was more effective than standard of care in reducing the severity of rickets, bowing or knock-knees, and tiredness and increased growth and the ability to walk further, in children with XLH.

There were more side effects considered related to burosumab than standard of care supplements. These side effects were most often related to injecting burosumab under the skin.

The results presented here are for one study. Other studies may provide new information or different results. Always talk to a doctor before making any treatment changes.

Further clinical research with burosumab is ongoing.

Where can I find more information about this study?

You can find more information about this study including a report of the study's results on these websites:

- https://clinicaltrials.gov/ct2/show/NCT02915705
- https://www.clinicaltrialsregister.eu/ctr/ Search: 2016-000600-29

If you have questions about the results, please speak with your doctor or staff at the study site.

The phone number for Ultragenyx is: 415-483-8800 and the email is: patientadvocacy@ultragenyx.com

Thank you

At Ultragenyx, our focus is developing medicines for people who live with rare and ultra-rare diseases. But it takes more than scientific knowledge and research to develop medications. Your involvement is essential and ensures that the research process moves forward. Thank you for your participation in this study and commitment to research.

ultragenyx pharmaceutical

Ultragenyx is a biopharmaceutical company committed to bringing products to patients for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases.

60 Leveroni Court • Novato, CA 94949
415-483-8800 • www.ultragenyx.com

Kyowa KIRIN

Kyowa Kirin commits to innovate drug discovery driven by state-of-the-art technologies. The company focuses on creating new values in the four therapeutic areas: nephrology, oncology, immunology/allergy and neurology.

www.kyowakirin.com

임상시험 결과

X염색체 유전성 저인산혈증(XLH)이 있는 소아 환자들에서 경구 인산염 및 활성 비타민 D 치료 대비 부로수맙(KRN23)의 유효성 및 안전성

감사 합니다!

임상시험의 시험대상자들은 전 세계 임상시험 참여자들의 거대한 커뮤니티에 소속되어 있습니다. KRN23으로도 불리는 부로수맙 관련 임상시험에 참여해 주신 아동과 부모님, 보호자 분들께 감사드립니다. 이 임상시험의 의뢰자인 Ultragenyx와 Kyowa Kirin은 참여한 분들께 감사함을 느끼고 있으며, 결과를 공유하는 것이 중요하다고 생각합니다.

이 요약은 어떤 내용인가요?

이 임상시험은 X염색체 유전성 저인산혈증(XLH)에 대한 표준 치료(경구 인산염 및 활성 비타민 D 병용)와 부로수맙을 비교하기 위해 실시되었습니다.

이 요약본은 UX023-CL301 임상시험의 주요 결과를 제시하며, 다음 사항에 대한 이해를 돕기 위해 제작되었습니다.

- XLH에 관한 이해
- 이 임상시험을 실시한 이유
- 이 임상시험의 참여 자격
- 이 임상시험의 시험대상자가 투여받은 약물
- 이 임상시험에서 측정한 결과
- 이 임상시험 기간 동안 보고된 부작용
- 이 임상시험이 XLH 보유자와 의료 사회에 기여한 내용
- 이 임상시험에 대한 추가 정보 확인할 수 있는 곳

이 문서를 담당 의사 또는 의료 서비스 제공자와 함께 검토하는 것이 도움이 될 수 있습니다.

임상시험 계획서 번호

UX023-CL301

NCT 번호

NCT02915705

연구 대상 치료제

- 부로수맙
- 경구 인산염 및 비타민 D

임상시험 의뢰자

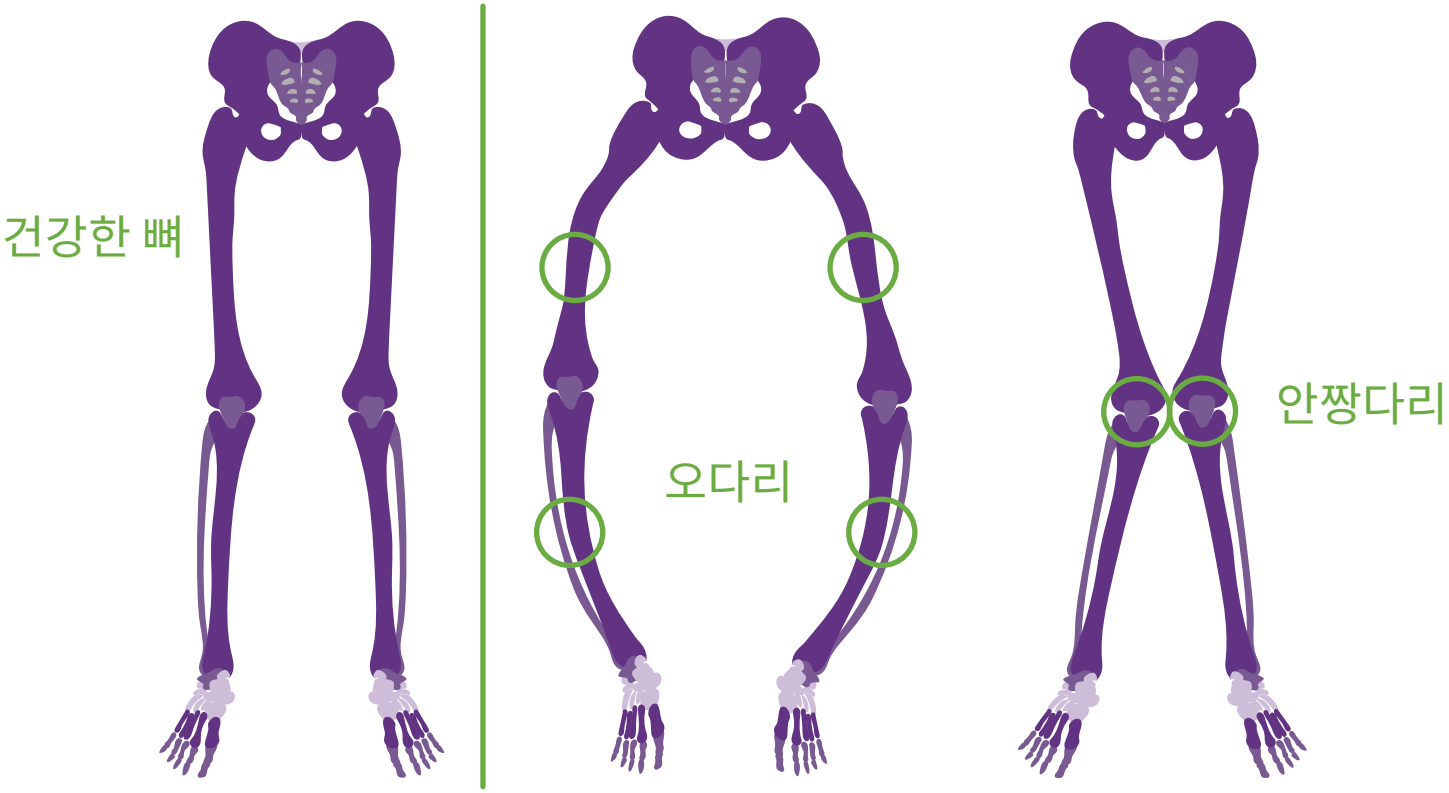
Ultragenyx 및 Kyowa Kirin

임상시험 날짜

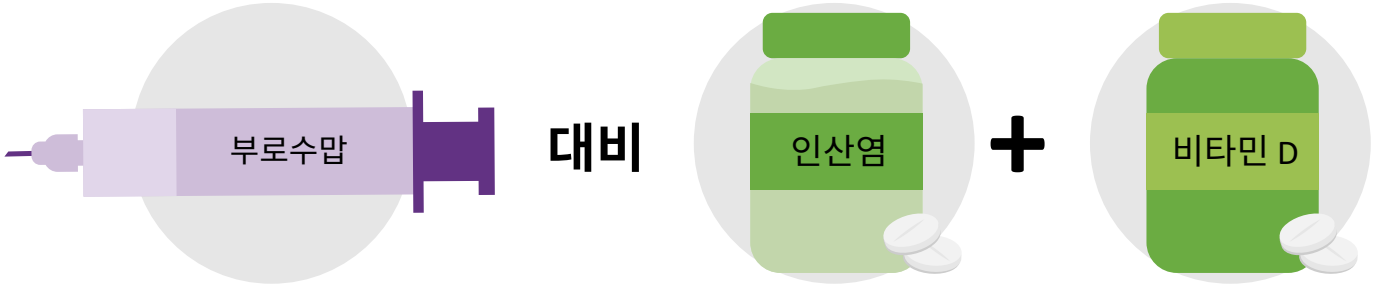
2016년 9월~ 2019년 7월

XLH란 무엇인가요?

XLH 질환으로 인해 신체의 인산염이 과도하게 손실됩니다. 인산염은 건강한 뼈 생성을 돕기 위해 필요합니다. 아동에서 이러한 인산염 수치 저하는 구루병이라는 뼈 질환을 유발할 수 있는데, 이 경우 뼈가 무르고 약해지며 다리 뼈가 굽어 서로 벌어지는 상태(오다리) 또는 서로 맞닿게 되는 상태(안짱다리)가 초래될 수 있습니다.



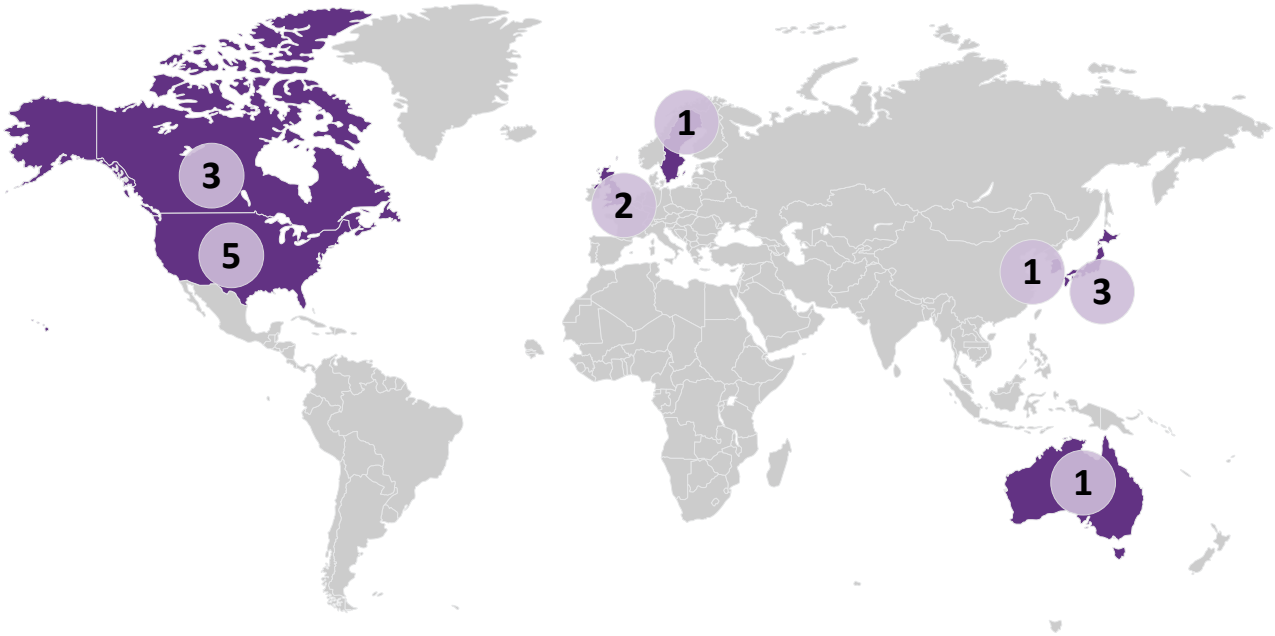
이 임상시험을 실시한 이유는 무엇인가요?



이 임상시험에서 연구자들은 부로수맙이 표준 치료(경구 인산염과 활성 비타민 D 병용)와 비교하여 얼마나 효과가 있는지 확인하고자 했습니다. 또한, 연구자들은 아동이 임상시험 기간 동안 경험하는 부작용이 있는지 알아보고자 했습니다. 부작용이란 의사들이 임상시험 치료제와 관련될 수 있다고 여기며 아동이 겪을 수 있는 원치 않거나 예상치 못한 경험입니다.

이 임상시험은 어디에서 실시했나요?

이 임상시험은 다음과 같은 16곳의 시험기관에서 실시했습니다: 미국 5곳, 일본 3곳, 캐나다 3곳, 영국 2곳, 스웨덴 1곳,한국 1곳, 호주 1곳.



이 임상시험에 누가 참여할 수 있나요?

XLH가 있는 아동이 특정 기준에 부합하면 이 임상시험에 참여할 수 있었습니다.
전체 기준은 https://clinicaltrials.gov/ct2/show/NCT02915705에서 확인할 수 있습니다.

아래는 임상시험 참여에 대한 몇 가지 주요 기준입니다.

X-선 측정에 따라 구루병이 있는 1~12세의 남아 또는 여아

특정한 혈중 인산염 수치 저하

아동이 XLH를 앓고 있음을 나타내는 실험실 검사 결과

일정 기간(아동의 연령에 기반) 동안 표준 치료 보충제를 투여받음

이 임상시험에서 아동이 받은 약물은 무엇인가요?

XLH가 있는 61명의 아동이 이 임상시험에 참여했습니다

임상시험 시작 전, 모든 아동은 **7일**간 표준 치료 보충제를 중단해야 했습니다. 이 단계는 임상시험 시작 시점에 아동의 신체에 표준 약물이 남아있지 않도록 하기 위해 실시되었습니다.

이 임상시험은 공개형 연구였습니다. 이는 모두가 어떤 약물을 투여 중인지 알고 있었지만, 아동이나 임상시험 의사 누구도 투여하는 치료제를 선택할 수 없었음을 의미합니다. 아동은 우연에 의해(무작위배정) 두 투여군 중 하나에 배정되었습니다. 이는 동전 던지기나 모자에서 숫자 뽑기와 같은 방식입니다.

부로수맙 투여군

시작 용량 0.8mg/kg

피하 주사로 2주마다 투여

이 투여군 내 인원: 29명의 아동(여아 16명, 남아 13명)

표준 치료 투여군

인산염 하루 다회 투여 및 활성 비타민 D 하루 1회 이상 투여

매일 경구 투여

이 투여군 내 인원: 32명의 아동(여아 18명, 남아 14명)

40주
결과 수집됨.

64주
결과 수집됨.

제64주에 일본과 한국의 모든 아동이 임상시험 중단

140주
결과 수집됨.

제64주에 임상시험이 연장되었으며 표준 치료 보충제 투여 아동은 부로수맙 투여로 전환했습니다.

기존의 부로수맙 투여군 아동은 계속해서 부로수맙을 투여받았습니다.

임상시험 결과에 대한 이해.

여기에는 연구자들이 답변드리고자 했던 주요 질문에 대한 결과 요약과, 연구자들이 생각하기에 아동과 보호자들이 관심을 가질 만한 다른 결과들이 포함되어 있습니다. 각 아동의 개별적인 결과는 다를 수 있으며 이 요약에는 포함되어 있지 않습니다. 귀하 또는 이 임상시험에 참여한 보호 아동이 개별 결과에 대해 궁금한 점이 있다면, 해당 시험기관의 의사 또는 직원에게 문의하시기 바랍니다.

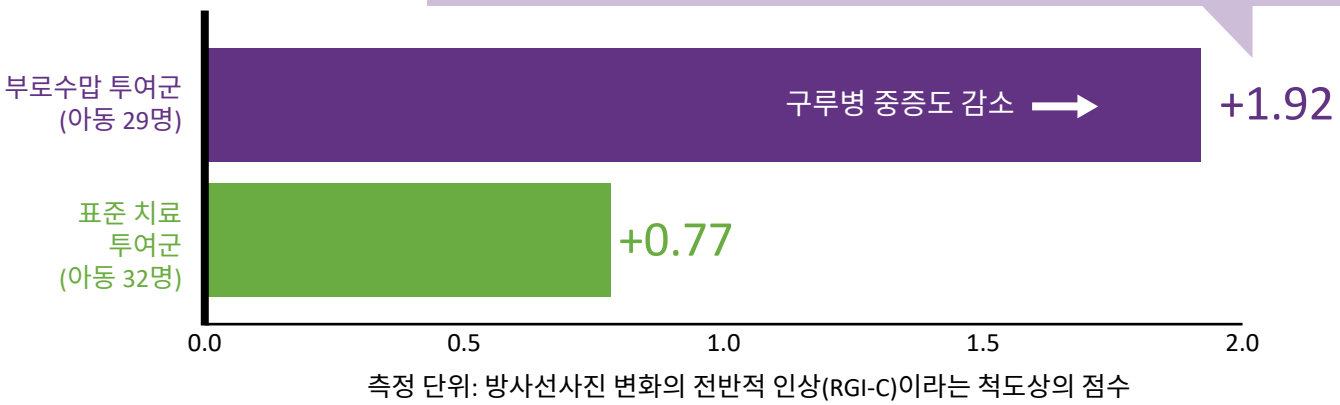
이 임상시험에서 측정한 주요 결과는 무엇이었나요?

제40주에 구루병의 변화 측정

XLH가 있는 아동에서 중요한 치료 목표는 뼈를 무르고 약하게 만드는 질환인 구루병을 치유하는 것입니다. 구루병의 변화를 측정하기 위해, 연구자들은 투여(부로수맙 또는 표준 치료) 시작 전후 아동의 손목 및 무릎 X-선을 비교하여 방사선사진 변화의 전반적 인상(RGI-C) 점수를 측정했습니다. 이 점수는 투여 시 치유, 악화 또는 변화 없음 여부를 측정합니다.

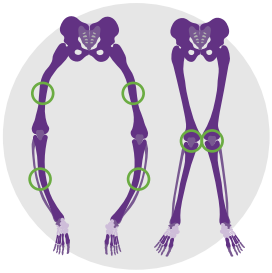
시험약은 제40주에 아동의 구루병에 어떤 영향을 미쳤나요?

제40주 시점에, 부로수맙을 투여받은 아동에서 표준 치료를 투여받은 아동에 비해 중증도가 더 많이 감소했습니다.



측정 단위: 방사선사진 변화의 전반적 인상(RGI-C)이라는 척도상의 점수

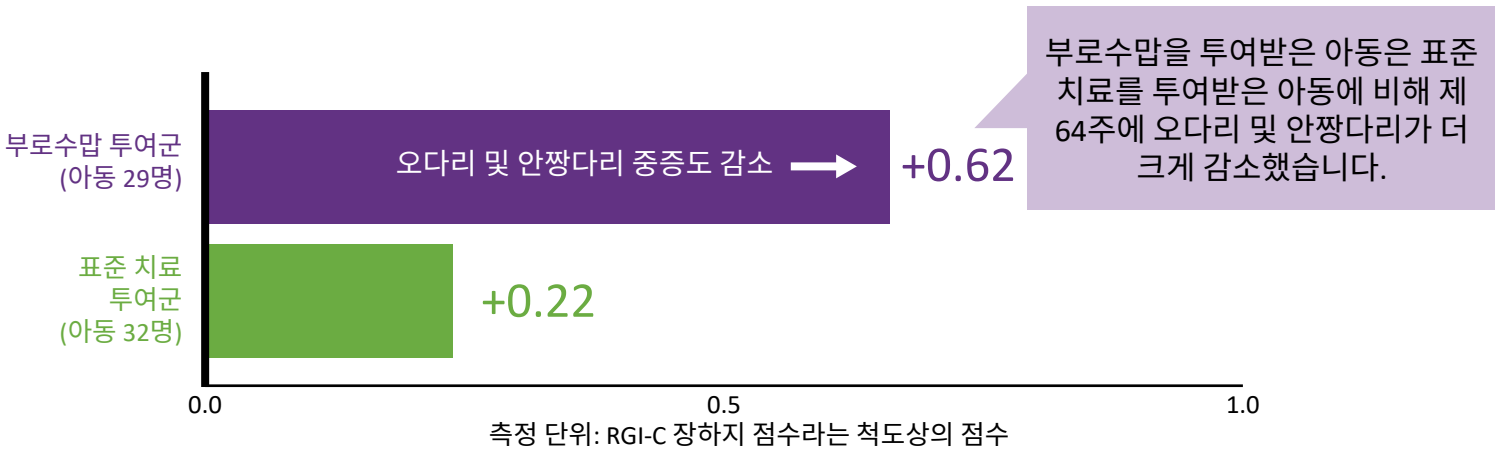
이 임상시험에서 측정한 다른 결과에는 무엇이 있었나요?



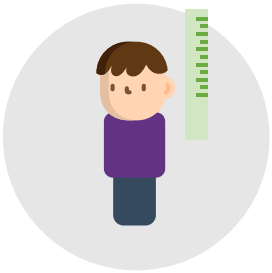
제64주에 오다리 및 안짱다리 측정

아동의 다리 X-선에서 얻은 RGI-C 점수 또한 다리의 오다리 및 안짱다리 중증도 측정에 사용되었습니다.

시험약은 제64주 시점에 다리 형태에 어떤 영향을 미쳤나요?



부로수맙을 투여받은 아동은 표준 치료를 투여받은 아동에 비해 제64주에 오다리 및 안짱다리가 더 크게 감소했습니다.

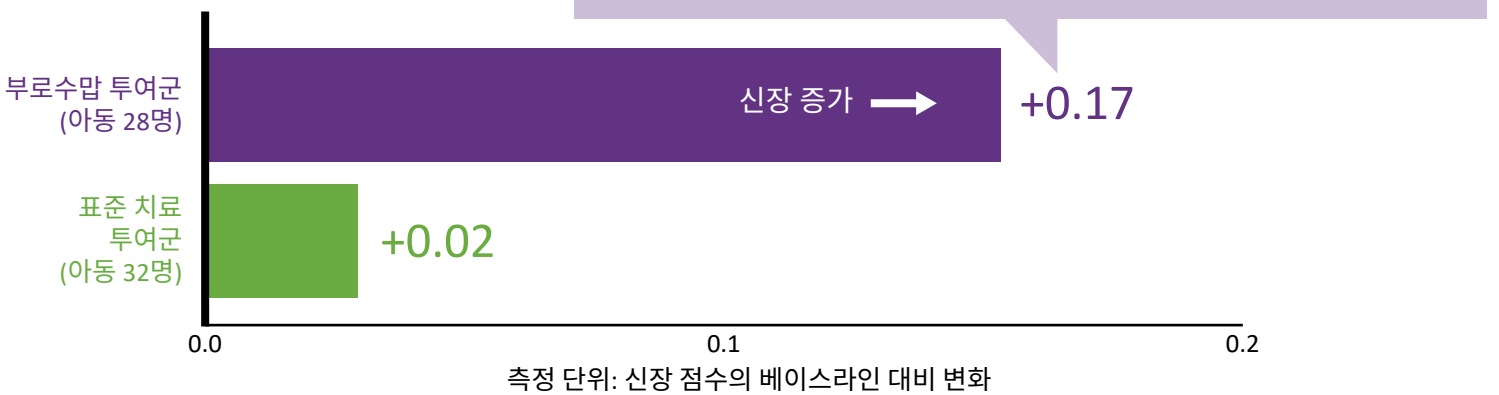


제64주에 아동의 신장 측정

신장을 측정하여 XLH를 앓지 않는 동일 연령 및 성별의 아동 신장과 비교했습니다. 이를 통해 신장 점수를 확보했습니다.

시험약이 제64주 신장 변화에 어떤 영향을 미쳤나요?

부로수맙을 투여받은 아동은 표준 치료를 투여받은 아동에 비해 제64주에 신장이 더 크게 증가했습니다.



이 임상시험에서 측정한 다른 결과에는 무엇이 있었나요?

제40주에 아동의 전반적 상태 측정

5세 이상의 아동을 대상으로 PROMIS(환자 보고 결과 측정 정보 시스템)라는 도구의 질문을 이용하여 운동성, 피로, 활동에 간섭하는 통증 정도 측면에서 아동의 상태를 측정했습니다.

시험약이 아동의 상태에 어떤 영향을 미쳤나요?

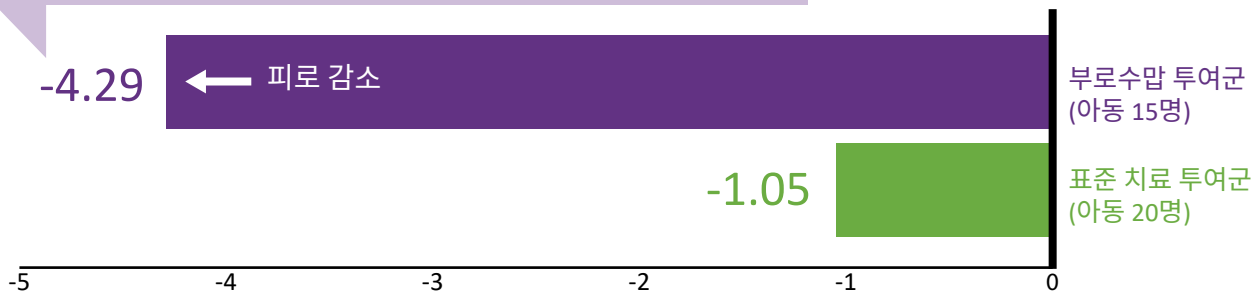
부로수맙을 투여받은 아동은 표준 치료를 투여받은 아동에 비해 제40주 보고에서 운동성이 더 크게 증가했습니다.

운동성 측정

측정 단위: PROMIS라는 척도에서 특히 운동성에 대한 점수의 베이스라인 대비 변화

부로수맙을 투여받은 아동은 표준 치료를 투여받은 아동에 비해 제40주 보고에서 피로가 더 크게 감소했습니다.

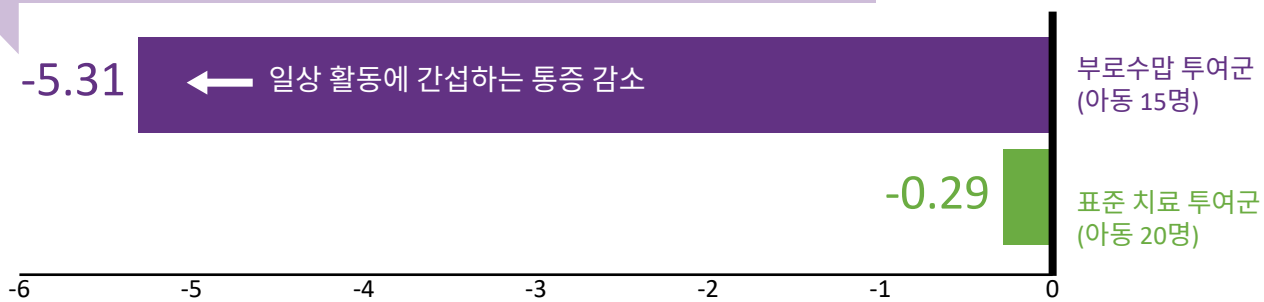
피로 측정



측정 단위: PROMIS라는 척도에서 특히 피로에 대한 점수의 베이스라인 대비 변화

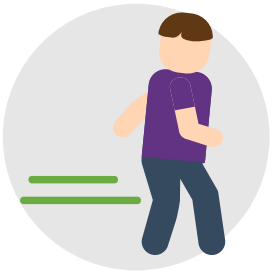
부로수맙을 투여받은 아동은 표준 치료를 투여받은 아동에 비해 제40주 보고에서 통증 간섭이 더 크게 감소했습니다.

통증 간섭 측정



측정 단위: PROMIS라는 척도에서 특히 통증에 대한 점수의 베이스라인 대비 변화

이 임상시험에서 측정한 다른 결과에는 무엇이 있었나요?

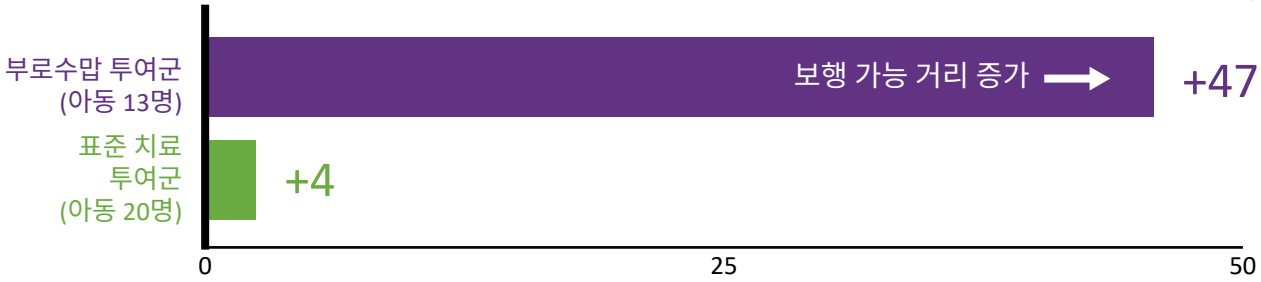


제40주에 아동의 보행 가능 거리 측정

이는 5세 이상의 아동을 대상으로 측정했습니다. 연구자들은 각 아동이 6분간 걸을 수 있는 거리를 측정했습니다.

시험약은 제40주에 아동의 6분간 보행 가능 거리에 어떤 영향을 미쳤나요?

부로수맙을 투여받은 아동은 표준 치료를 투여받은 아동에 비해 제40주에 6분간 보행 가능 거리가 더 크게 증가한 것으로 나타났습니다.



측정 단위: 6분간 보행한 미터 수의 베이스라인 대비 변화(1미터 = 3.28피트)

이 임상시험의 제64주까지 어떤 부작용이 보고되었나요?

부작용은 본 임상시험에서 투여한 약물과 관련성이 있을 수도, 없을 수도 있습니다. 뿐만 아니라 부작용은 임상시험 중 투여받은 다른 약물이나 아동의 다른 의학적 상태, 또는 임상시험에서 실시한 어떤 검사와 관련성이 있을 수도 있습니다.

본 항에서는 이 임상시험 기간 중 발생하였으며 의사들이 시험약 투여와 관련될 수 있다고 여긴 부작용을 요약합니다.

부작용은 사망을 초래하거나 생명을 위협하거나, 선천적 결손 또는 장애를 유발하거나, 병원 치료가 필요한 경우 “중대한” 것으로 간주됩니다.

이 임상시험의 제64주까지 어떤 부작용이 보고되었나요?

시험약 투여와 관련 가능성이 있는 부작용		부작용을 경험한 것으로 보고된 아동의 수
 부작용을 경험한 아동은 몇 명인가요? (중대한 것으로 여겨지는 부작용을 포함하여 아동에서 보고된 모든 부작용)	**부로수맙**	아동 29명 중 **17명**(58.6%)
	표준 치료	아동 32명 중 **8명**(25.0%)
 중대한 부작용을 경험한 아동은 몇 명인가요?	**부로수맙**	아동 29명 중 **0명**(0%)
	표준 치료	아동 32명 중 **0명**(0%)

이 임상시험 기간 동안 사망하거나 부작용 또는 중대한 부작용으로 인해 시험약 투여를 중단한 아동은 없습니다.

이 임상시험의 제64주까지 어떤 부작용이 보고되었나요?

아래 그래프는 제64주까지 보고된 다음과 같은 부작용을 나타냅니다.

- 의사들이 생각하기에 시험약과 관련성이 있을 수 있는 부작용
- 이 임상시험에 참여한 모든 아동 중 10%를 초과해 발생한 부작용
- 다른 부작용도 있었으나, 발생 아동 수가 더 적음

시험약 투여와 관련 가능성이 있는 부작용

부작용을 경험한 것으로 보고된 아동의 수

부로수맙 = 영향을 받은 아동 = 영향을 받지 않은 아동

표준 치료 = 영향을 받은 아동 = 영향을 받지 않은 아동

주사 부위의 피부 발적

아동 29명 중 **8명**(27.6%)

아동 32명 중 **0명**(0%)

팔, 손, 다리 또는 발의 통증

아동 29명 중 **7명**(24.1%)

아동 32명 중 **2명**(6.3%)

주사 부위의 피부 반응

아동 29명 중 **7명**(24.1%)

아동 32명 중 **0명**(0%)

관절의 통증

아동 29명 중 **6명**(20.7%)

아동 32명 중 **4명**(12.5%)

치아 감염

아동 29명 중 **4명**(13.8%)

아동 32명 중 **0명**(0%)

주사 부위의 가려움증

아동 29명 중 **3명**(10.3%)

아동 32명 중 **0명**(0%)

주사 부위의 발진

아동 29명 중 **3명**(10.3%)

아동 32명 중 **0명**(0%)

주사 부위의 부기

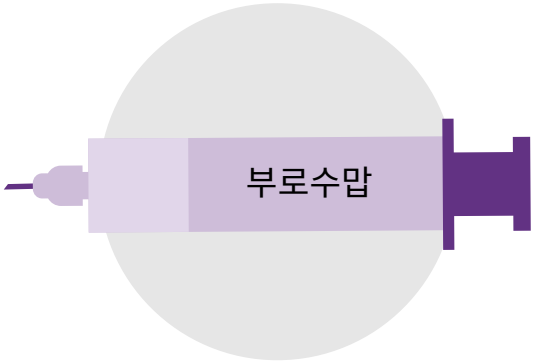
아동 29명 중 **3명**(10.3%)

아동 32명 중 **0명**(0%)

이 정보를 읽을 때, 표준 치료는 주사가 아니라 경구로 투여했다는 점을 잊지 않아야 합니다.

제64주 이후, 표준 치료를 투여받았던 아동은 부로수맙으로 전환하여 최대 제140주 동안 투여를 받았습니다. 부로수맙을 투여받고 있던 아동은 최대 제140주 동안 계속해서 부로수맙을 투여받았습니다. 전반적으로, 부작용은 첫 64주 기간과 동일했습니다.

이 임상시험은 XLH 보유자와 연구자들에게 어떤 도움이 되었나요?



전체적으로, 연구자들은 XLH가 있는 아동에서 부로수맙이 표준 치료에 비해 구로병과 오다리 또는 안짱다리, 그리고 피로의 중증도 감소, 성장 증가, 보행 가능 거리 증가에 더 효과적이었음을 확인했습니다.

표준 치료 보충제보다 부로수맙과 관련된 것으로 간주된 부작용이 더 많았습니다. 이러한 부작용은 부로수맙의 피하 주사와 관련된 경우가 가장 빈번했습니다.

여기에 제시된 결과는 하나의 임상시험에 관련된 것입니다. 다른 임상시험에서는 새로운 정보 또는 다른 결과가 제공될 수 있습니다. 일체의 치료 변경을 실시하기에 앞서 항상 의사와 상의하시기 바랍니다.

부로수맙에 대한 임상시험이 추가로 진행 중입니다.

이 임상시험에 대한 추가 정보는 어디에서 확인할 수 있나요?

아래 웹사이트에서 임상시험 결과 보고를 포함하여 이 임상시험에 대한 추가 정보를 확인할 수 있습니다.

- https://clinicaltrials.gov/ct2/show/NCT02915705
- https://www.clinicaltrialsregister.eu/ctr/ Search: 2016-000600-29

결과에 대해 궁금한 점이 있는 경우, 시험기관의 담당 의사나 직원에게 문의하시기 바랍니다.

Ultragenyx 전화번호: 415-483-8800, 이메일: patientadvocacy@ultragenyx.com

감사합니다

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Kyowa KIRIN

Kyowa Kirin은 최첨단 기술을 바탕으로 약물 발견 혁신화를 위해 노력하고 있습니다. 이 기업에서는 신장학, 종양학, 면역학/알레르기, 신경학의 네 가지 치료 분야에서 새로운 가치를 창출하는 데 주력합니다.

www.kyowakirin.com

Version 1.0 03/2020

MRCC-KRN23-00133

Kliniska studieresultat

Effekt och säkerhet av Burosumab (KRN23) jämfört med oralt fosfat och aktiv D-vitaminbehandling hos pediatriska patienter med X-bunden hypofosfatemisk rakit (XLH)

Tack!

Deltagare i kliniska studier tillhör en stor grupp av människor som deltar i klinisk forskning runt hela världen. Tack till barn, föräldrar, och vårdgivare som deltog i den kliniska studien av burosumab, som även kallas KRN23. Ultragenyx och Kyowa Kirin, sponsorerna för denna studie, är tacksamma för dem som deltog och anser att det är viktigt att dela resultaten.

Vad täcker denna sammanfattning?

Denna studie genomfördes för att jämföra burosumab med vårdstandarden (en kombination av oralt fosfat och aktivt D-vitamin) för X-bunden hypofosfatemisk rakit (XLH).

Denna sammanfattning visar huvudresultaten från UX023-CL301-studien, och skapades för att hjälpa personer att få reda på:

- Vad XLH är
- Varför denna studie genomfördes
- Vem fick delta i denna studie
- Vilka mediciner deltagare i denna studie fick
- Vilka resultat som mättes i denna studie
- Vilka biverkningar som rapporterades under denna studie
- Hur denna studie hjälpte personer med XLH och sjukvården
- Var man hittar mer information om denna studie

Det kan vara användbart att granska detta dokument med din läkare eller vårdgivare.

Protokollnummer:

UX023-CL301

NCT-nummer

NCT02915705

Behandlingar som studerats

- Burosumab
- Oralt fosfat och vitamin D

Studiens sponsorer

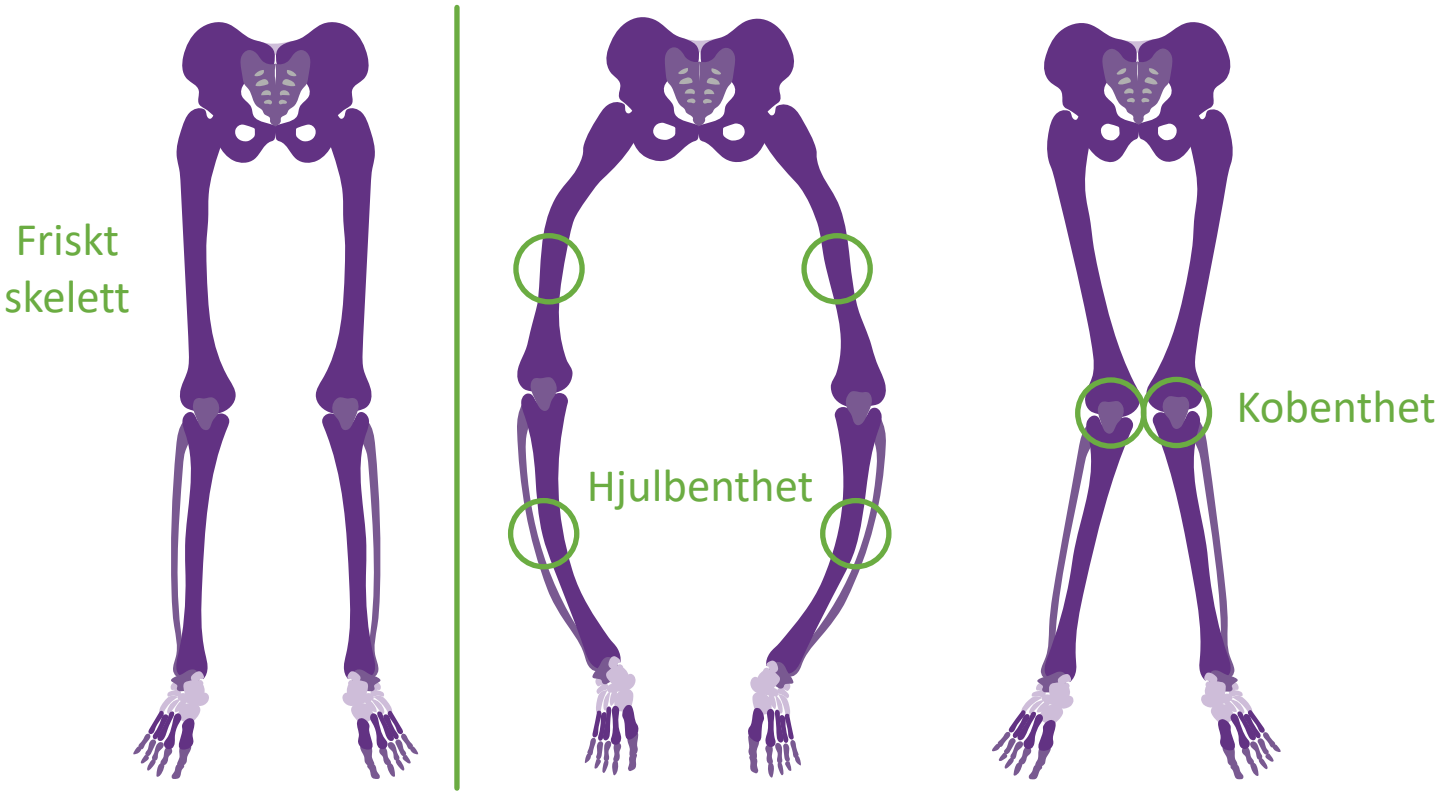
Ultragenyx och Kyowa Kirin

Studiedatum

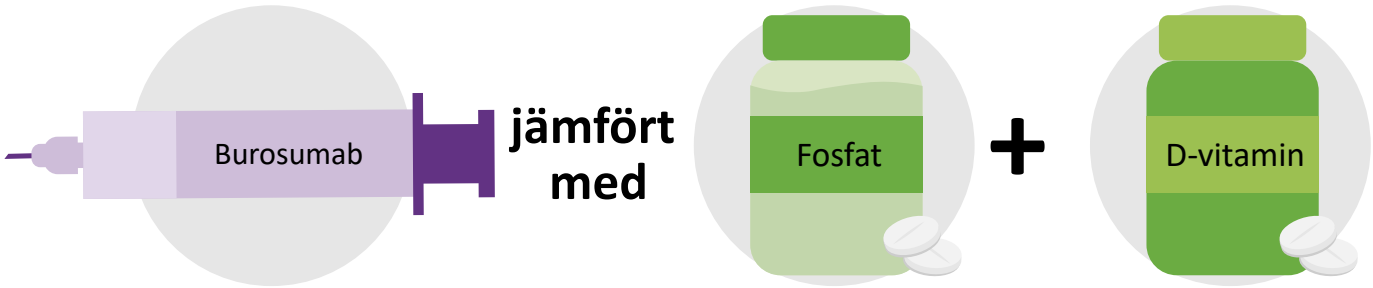
september 2016 till juli 2019

Vad är XLH?

XLH är en sällsynt och livslång bensjukdom som påverkar livet för barn och vuxna. XLH är en sjukdom där kroppen förlorar för mycket fosfat. Fosfat är nödvändigt för att kunna skapa friskt skelett. Hos barn kan dessa låga nivåer av fosfat orsaka ett skelettillstånd som kallas rakit, där skelettet blir mjukt och svagt och kan göra att skelettet i benen böjs bort från varandra (hjulbenthet) eller böjs mot varandra (kobenthet).



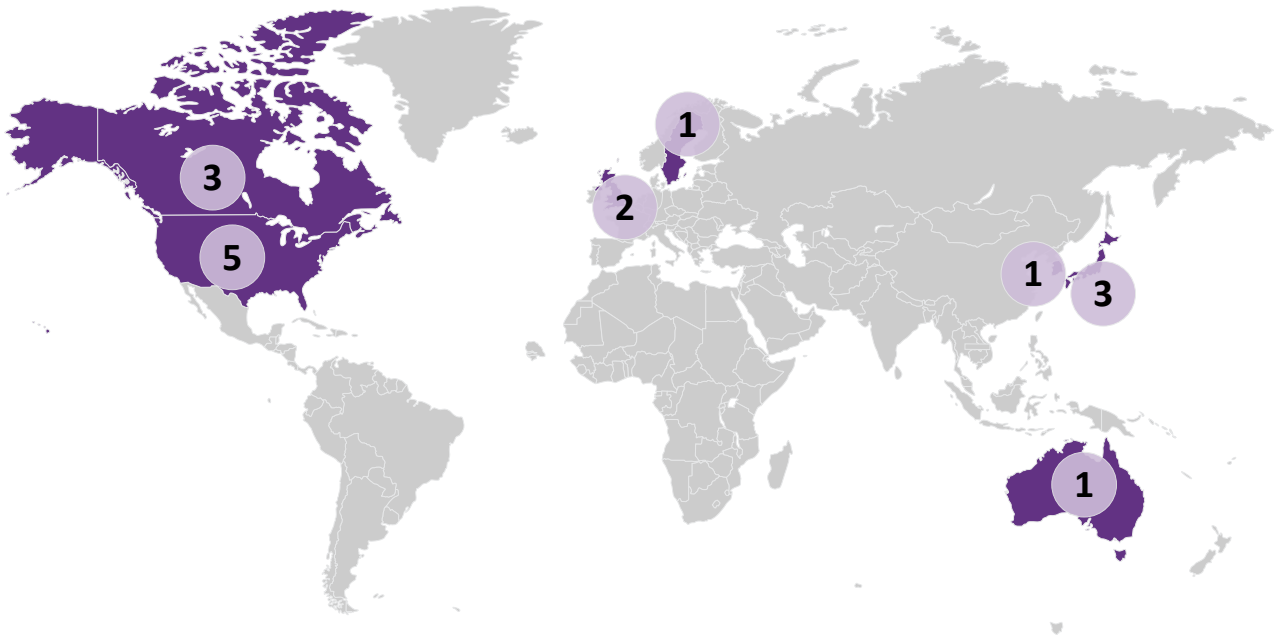
Varför utfördes denna studie?



I denna studie ville forskarna se hur väl burosumab fungerade jämfört med vårdstandarden (en kombination av oralt fosfat och aktivt D-vitamin). Forskarna ville också ta reda på om barnen hade några biverkningar under studien. Biverkningar är oönskade eller oväntade upplevelser som barn kan ha, som läkarna tror kan vara förknippade med studiebehandlingen.

Var utfördes denna studie?

Denna studie ägde rum på 16 kliniker: 5 i USA, 3 i Japan, 3 i Kanada, 2 i Storbritannien, 1 i Sverige, 1 i Sydkorea, och 1 i Australien.



Vem fick delta i denna studie?

Barn med XLH kunde delta i denna studie om de uppfyllde specifika kriterier. Samtliga kriterier finns här: https://clinicaltrials.gov/ct2/show/NCT02915705.

Nedan visas några huvudkriterier för vem kunde delta i studien:

Pojkar eller flickor, i åldern 1 till 12 år, med rakit, enligt vad som fastställs av röntgen

Specifika låga nivåer av fosfat (phosphate) i blodet

Laboratorieprovsvar som visar att barn hade XLH

Fick standardvårdtillskott under en viss tid (baserat på barnets ålder)

Vilka läkemedel fick barnen i denna studie?

61

barn med XLH deltog i denna studie

Innan början av studien, var alla barn tvungna att sluta med vårdstandardtillskott i **7 dagar**. Detta steg vidtogs för att säkerställa att ingen mängd standardläkemedel fanns kvar i barnens kroppar i början av studien.

Detta var en öppen studie. Detta innebär att alla visste vilket läkemedel de tog men varken barnet eller studieläkaren kunde välja vilken behandling de fick. Barn tilldelades slumpmässigt till en av två behandlingsgrupper (randomiserades). Detta är som att singla slant eller dra lott.

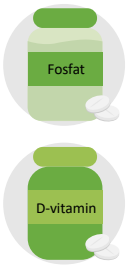
Burosumab-grupp

0,8 mg/kg startdos

Ges varannan vecka som en injektion under huden

Det fanns:
29 barn
(16 flickor, 13 pojkar)
i denna grupp

Vårdstandardgruppen



Flera doser per dag av fosfat och en eller flera dagliga doser av aktivt D-vitamin

Ges dagligen via munnen

Det fanns:
32 barn
(18 flickor, 14 pojkar)
i denna grupp

40 veckor

Resultat samlades in.

64 veckor

Resultat samlades in.

Alla barn i Japan och Korea avbröt sitt deltagande i studien vid Vecka 64

Vid Vecka 64 utökades studien och barn som fick vårdstandardtillskott växlade till att få burosumab

Barn som redan var i burosumab-gruppen fortsatte att få burosumab

140 veckor

Resultat samlades in.

Förstå studieresultaten.

Detta är en resultatsammanfattning av huvudfrågan som forskarna ville besvara, och andra resultat som forskare tror kan vara intressanta för barnen och deras vårdgivare. Individuella resultat för varje barn kan vara annorlunda och inkluderas inte i denna sammanfattning. Om du eller barnet i din vård som deltagit i denna studie har frågor om individuella resultat, ska du kontakta läkaren eller personalen på din studieklinik.

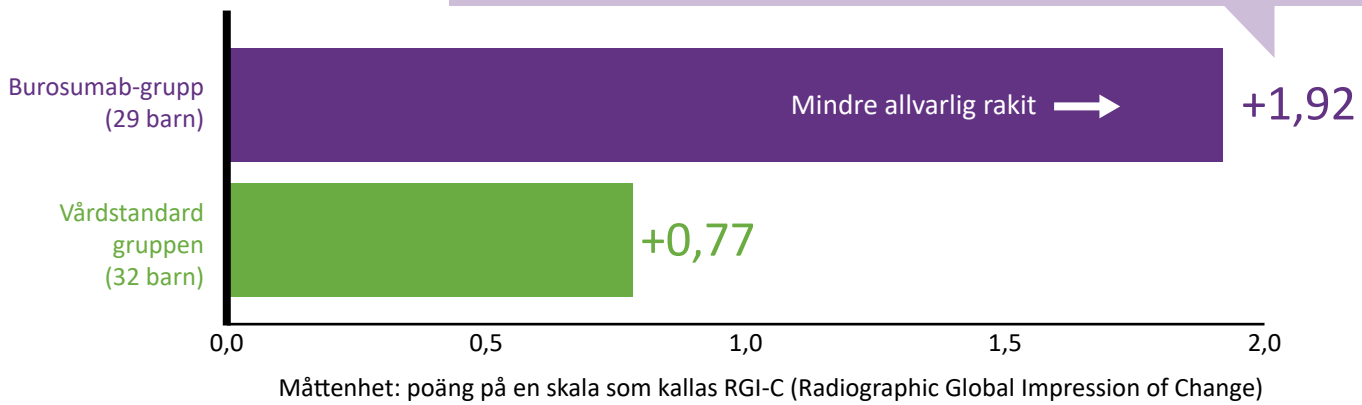
Vilken var det huvudsakliga resultatet som mättes i denna studie?

Mäter förändringen av rakit vid Vecka 40

Ett viktigt behandlingsmål hos barn med XLH är att läka rakit, ett tillstånd där skelettet blir mjukt och svagt. För att mäta förändringar i rakit, jämförde forskarna röntgenbilder av barnens handleder och knän, före och efter påbörjad behandling (burosumab eller vårdstandarden) för att fastställa RGI-C-poängen (Radiographic Global Impression of Change). Detta resultat mäter om behandlingen medförde en läkning, förvärring eller ingen förändring.

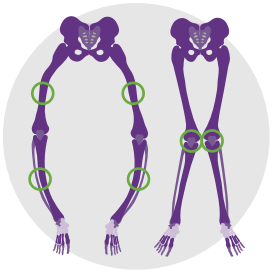
Hur påverkade studieläkemedlet ett barns rakit vid Vecka 40?

Svårighetsgraden av rakit minskade mer hos barn som fick burosumab jämfört med barn som behandlades med vårdstandarden vid Vecka 40.



Måttenhet: poäng på en skala som kallas RGI-C (Radiographic Global Impression of Change)

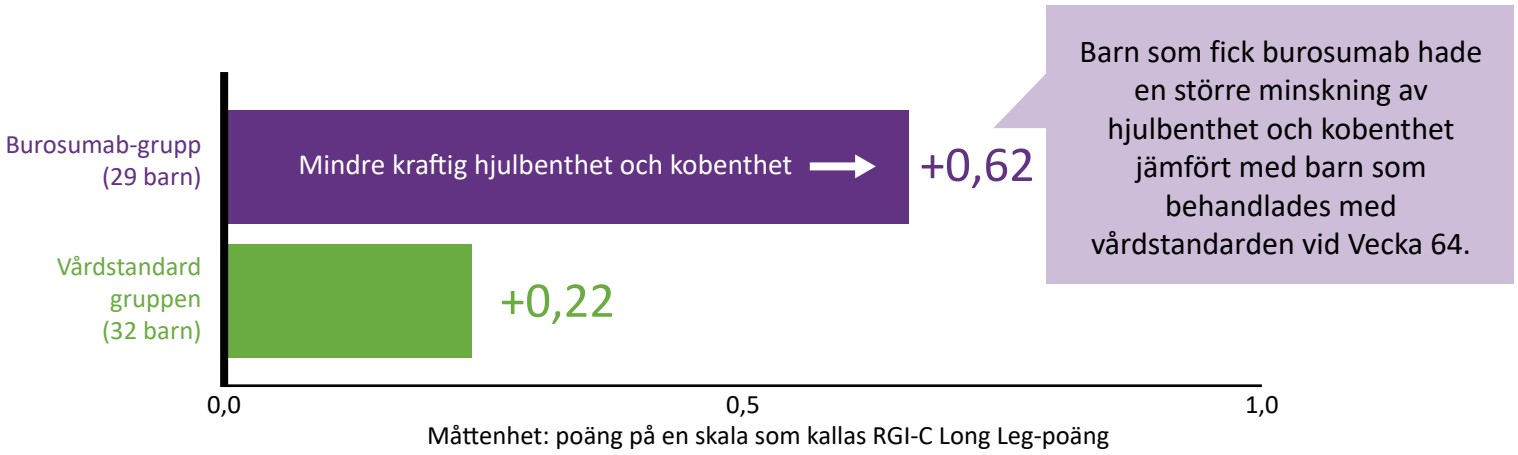
Vilka andra resultat mättes i denna studie?



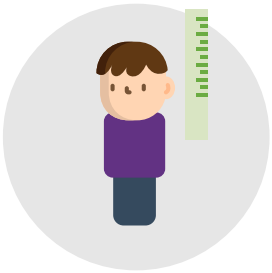
Mätning av hjulbenthet och kobenthet vid Vecka 64

RGI-C poängen från röntgen av ben hos barn användes också för att mäta svårighetsgraden av hjulbenthet och kobenthet.

Hur påverkade studieläkemedlet formen av benen vid Vecka 64?



Barn som fick burosumab hade en större minskning av hjulbenthet och kobenthet jämfört med barn som behandlades med vårdstandarden vid Vecka 64.

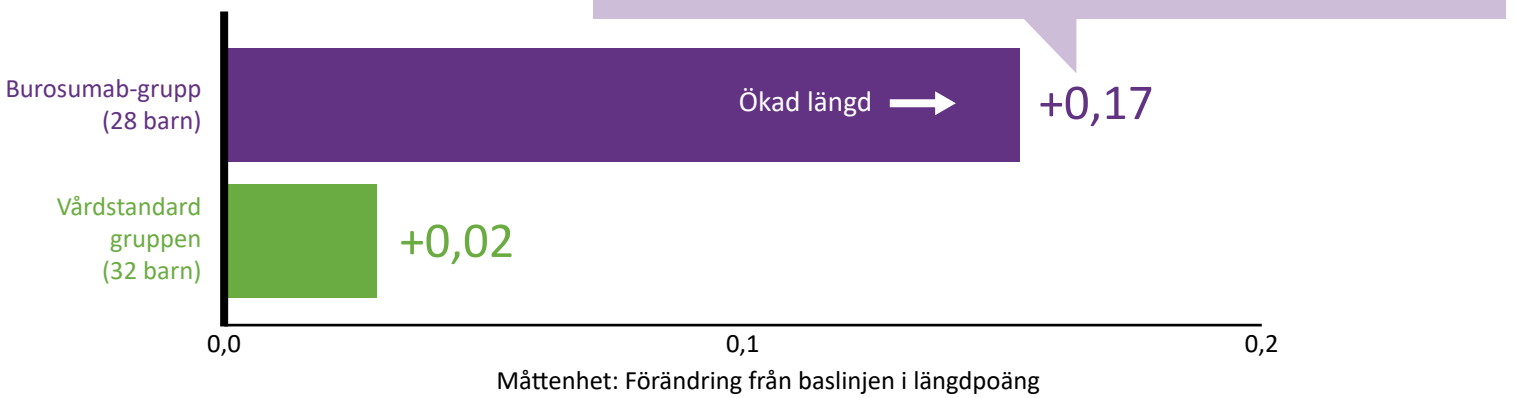


Mätning av längden av barn vid Vecka 64

Längden mättes och jämfördes med längden hos barn i samma ålder och kön, som inte hade XLH. Detta gav en längdpoäng.

Hur påverkade studieläkemedlet en förändring av längd vid Vecka 64?

Barn som fick burosumab hade en större ökning av längd jämfört med barn som behandlades med vårdstandarden vid Vecka 64.



Vilka andra resultat mättes i denna studie?

Mätning av ett barns allmänna välbefinnande vid Vecka 40

Frågor från ett verktyg som heter PROMIS (Patient-Reported Outcomes Measurement Information System) användes för barn från 5 års ålder för att mäta deras välbefinnande när det gäller rörelseförmåga, trötthet och hur mycket smärta störde aktiviteter.

Hur påverkade studieläkemedlet barns välbefinnande?

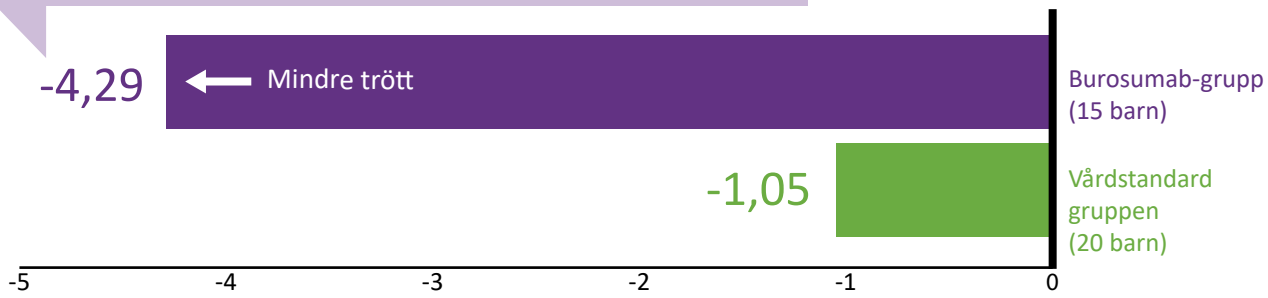
En ökning av rörlighet rapporterades hos barn som fick burosumab jämfört med barn som behandlades med vårdstandarden vid Vecka 40.

Mätning av rörlighet

Måttenhet: Förändring från baslinjen i poäng på en skala som kallas PROMIS, specifikt för rörlighet

En minskning av trötthet rapporterades hos barn som fick burosumab jämfört med barn som behandlades med vårdstandarden vid Vecka 40.

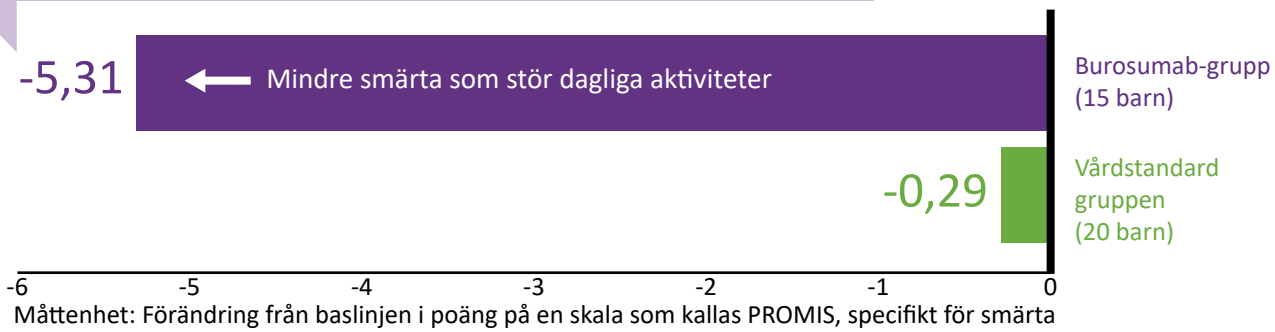
Mätning av trötthet



Måttenhet: Förändring från baslinjen i poäng på en skala som kallas PROMIS, specifikt för trötthet

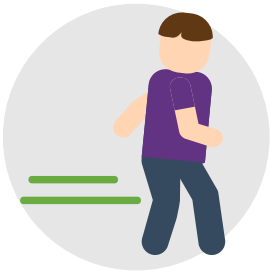
En större minskning av smärtstörning rapporterades hos barn som fick burosumab jämfört med barn som behandlades med vårdstandarden vid Vecka 40.

Mätning av smärtstörning



Måttenhet: Förändring från baslinjen i poäng på en skala som kallas PROMIS, specifikt för smärta

Vilka andra resultat mättes i denna studie?

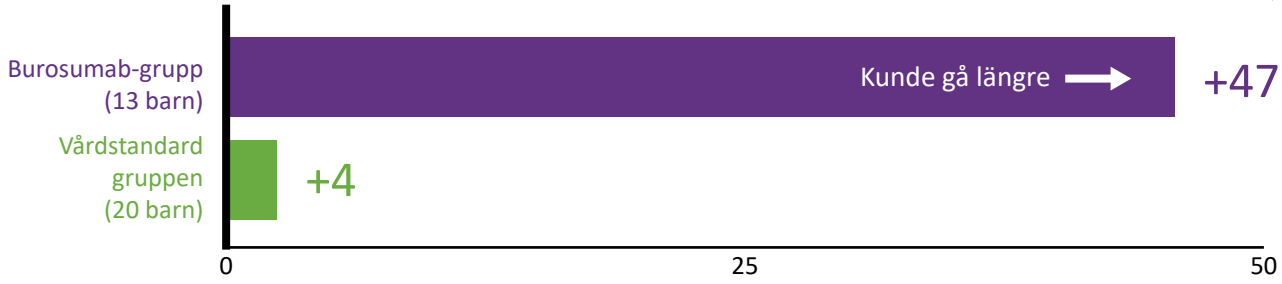


Mätning av hur långt ett barn kunde gå vid Vecka 40

Detta mättes hos barn i åldern 5 år eller äldre. Forskarna mätte avståndet som varje barn kunde gå på 6 minuter.

Hur påverkade studieläkemedlet hur långt ett barn kunde gå på 6 minuter vid Vecka 40?

Barn som fick burosumab visade en större ökning av hur långt de kunde gå på 6 minuter, jämfört med barn som fick vårdstandarden vid Vecka 40.



Måttenhet: Förändring från baslinjen i antal meter som barnen gick på 6 minuter

Vilka biverkningar rapporterades upp till Vecka 64 i denna studie?

Biverkningar kan vara eller eventuellt inte vara förknippade med de läkemedel som ges i studien. Biverkningar kan också vara förknippade med andra läkemedel som tas under deltagande i studien, med barnets andra medicinska tillstånd, eller med eventuella tester som utförs i studien.

Detta avsnitt är en sammanfattning av de biverkningar som förekom under denna studie, som läkarna trodde kunde vara förknippade med studieläkemedlet som togs.

En biverkning anses vara ”allvarlig” när den orsakar dödsfall eller är livshotande, orsakar en medfödd missbildning eller funktionsnedsättning, eller kräver sjukhusvård.

Vilka biverkningar rapporterades upp till Vecka 64 i denna studie?

Biverkningar som möjligtvis förknippas med behandlingen		Antalet barn som rapporterades ha biverkningar
 Hur många barn fick biverkningar? (alla biverkningar som rapporterades av barn, inklusive de biverkningar som ansågs vara allvarliga)	**Burosumab**	**17 barn av 29 (58,6 %)**
	Vårdstandarden	**8 barn av 32 (25,0 %)**
 Hur många barn fick allvarliga biverkningar?	**Burosumab**	**0 barn av 29 (0 %)**
	Vårdstandarden	**0 barn av 32 (0 %)**

Inget barn dog under denna studie eller slutade ta studiebehandlingen på grund av biverkningar eller allvarliga biverkningar.

Vilka biverkningar rapporterades upp till Vecka 64 i denna studie?

Bilden nedan visar biverkningarna som rapporterats fram till Vecka 64, som:

- läkarna trodde kunde vara relaterade till studieläkemedlet
- inträffade hos mer än 10 % av alla barn i studien
- det förekom andra biverkningar, men dessa drabbade färre barn

Biverkningar som möjligtvis förknippas med behandlingen

Antal barn som rapporterades ha biverkningar

Burosumab = barn som påverkades = barn som inte påverkades

Vårdstandarden = barn som påverkades = barn som inte påverkades

Rodnad av huden på injektionsstället

8 barn av 29 (27,6 %)

0 barn av 32 (0 %)

Smärta i armar, händer, ben eller fötter

7 barn av 29 (24,1 %)

2 barn av 32 (6,3 %)

Hudreaktion vid injektionsstället

7 barn av 29 (24,1 %)

0 barn av 32 (0 %)

Smärta i leder

6 barn av 29 (20,7 %)

4 barn av 32 (12,5 %)

Infektion i tänder

4 barn av 29 (13,8 %)

0 barn av 32 (0 %)

Klåda vid injektionsstället

3 barn av 29 (10,3 %)

0 barn av 32 (0 %)

Hudutslag vid injektionsstället

3 barn av 29 (10,3 %)

0 barn av 32 (0 %)

Svullnad vid injektionsstället

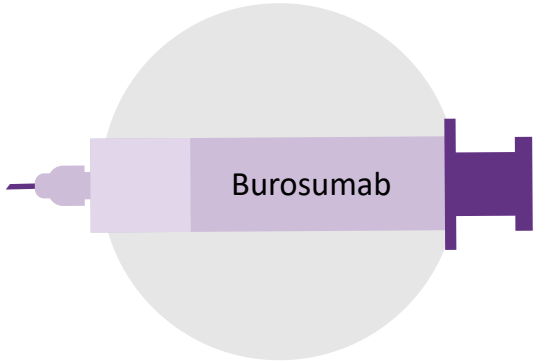
3 barn av 29 (10,3 %)

0 barn av 32 (0 %)

När du läser denna information, är det viktigt att komma ihåg att vårdstandarden gavs via munnen, och inte som en injektion.

Efter Vecka 64, växlade barn som fick vårdstandarden till att få burosumab upp till Vecka 140. Barn som tagit burosumab fortsatte att ta burosumab upp till Vecka 140. Totalt sett var biverkningarna desamma som under de första 64 veckorna.

Hur har den här studien hjälpt människor med XLH och forskare?



Totalt sett fann forskarna att burosumab var mer effektivt än vårdstandarden för att minska svårighetsgraden av rakit, hjulbenthet eller kobenthet, samt trötthet och ökad tillväxt och förmågan att gå längre, hos barn med XLH.

Fler biverkningar ansågs vara förknippade med burosumab än vårdstandardtillskott. Dessa biverkningar var oftast relaterade till injektion av burosumab under huden.

Resultaten som presenteras här är för en studie. Andra studier kan ge ny information eller annorlunda resultat. Tala alltid med en läkare innan du gör några behandlingsförändringar.

Ytterligare klinisk forskning med burosumab pågår.

Var kan jag få mer information om denna studie?

Du hittar mer information om denna studie, inklusive en rapport om studiens resultat, på dessa webbplatser:

- https://clinicaltrials.gov/ct2/show/NCT02915705
- https://www.clinicaltrialsregister.eu/ctr/ Search: 2016-000600-29

Om du har frågor om resultaten, tala med din läkare eller personalen på studiekliniken.

Telefonnumret till Ultragenyx är: +1 415 483 8800, och e-postadressen är: patientadvocacy@ultragenyx.com

Tack!

Hos Ultragenyx fokuserar vi på att utveckla läkemedel för personer som lever med sällsynta och mycket sällsynta sjukdomar. Men det krävs mer än vetenskaplig kunskap och forskning för att utveckla läkemedel. Ditt engagemang är väsentligt och säkerställer att forskningsprocessen går framåt. Tack för ditt deltagande i denna studie och ditt engagemang i forskningen.

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Kyowa Kirin

Kyowa Kirin engagerar sig i att förnya läkemedelsupptäckter som drivs av toppmodern teknik. Företaget fokuserar på att skapa nya användningar inom de fyra terapiområdena: nefrologi, onkologi, immunologi/allergi och neurologi.

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