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Journal

Journal of Feline Medicine and Surgery Open Reports, 10(2)

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Publication Date

2024

DOI

10.1177/20551169241254227

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Feline dystrophin-deficient muscular dystrophy misdiagnosed as *Toxoplasma myositis*

Journal of Feline Medicine and Surgery Open Reports
1–7

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DOI: 10.1177/20551169241254227

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Abstract

Case summary A 6-month-old male entire domestic shorthair cat presented for presumptive *Toxoplasma* myopathy that was non-responsive to antiprotozoal therapy. Clinical features included marked macroglossia, dysphagia, regurgitation, truncal muscle hypertrophy, pelvic limb gait abnormalities and megaesophagus. Relevant diagnostics included serial creatine kinase activity, cardiac troponin I, fluoroscopic swallow study and routine muscle histopathology. Ultimately, post-mortem histopathology with immunostaining demonstrated markedly decreased or absent staining for the rod and carboxy terminus of dystrophin, confirming a dystrophin-deficient muscular dystrophy (MD). The misdiagnosis of toxoplasmosis was based on an increased IgG titre and muscle histopathology submitted to a local laboratory. Treatment for megaesophagus included vertical feeding of wet food only, sildenafil and omeprazole. Dysphagia and regurgitation improved moderately. Presumptive hyperaesthesia and muscle pain were managed with anti-inflammatory doses of prednisolone. The patient was ultimately euthanased as a result of progressive MD signs and uraemia at 2 years of age.

Relevance and novel information This case report highlights the collective clinical features of MD, as they could be considered pathognomonic for this rare condition and must be differentiated from other myopathies via specific immunostaining of muscle biopsies. This is crucial to obtain a correct and early diagnosis, allowing instigation of potentially valuable treatments. Megaesophagus is an inconsistent feature in feline MD in addition to the more commonly observed oropharyngeal dysphagia. Management with a canned diet, sildenafil, omeprazole and upright feeding was beneficial with moderate improvement in the frequency of regurgitation. Prednisolone was thought to minimise the presumptive myalgia.

Keywords: Megaesophagus; muscular dystrophy; hyperCKaemia; oropharyngeal dysphagia; macroglossia; lingual hypertrophy; calcinosis circumscripta; muscular hypertrophy; dystrophin deficient

Accepted: 25 April 2024

Introduction

Muscular dystrophies (MDs) are a heterogeneous group of genetic diseases that result in progressive muscle degeneration, cycles of regeneration and progressive weakness. Three forms of MD have been identified in cats, including feline X-linked dystrophin deficiency (Maine Coon and domestic shorthair [DSH]),^{1–4} merosin (laminin alpha [α] 2) deficiency (Siamese, Maine Coon, DSH)^{3–6} and sarcoglycan deficiency (DSH).^{3,7}

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Mutations of genes coding for dystrophin or its associated proteins cause disruption of the dystrophin–glycoprotein complex (Figure 1), leading to membrane instability and leakage of intracellular contents, including creatine kinase (CK), into the extracellular fluid space, alongside skeletal and cardiac muscle fibre damage secondary to an influx of intracellular calcium and protease activation leading to myocyte death. Loss of CK prevents the formation of phosphocreatine, which is used as energy by the myocytes, leading to weakness.^{3,9}

Common clinical signs of MD in cats include a stilted gait, weakness, dysphagia, regurgitation, muscle atrophy or hypertrophy and, in particular, macroglossia, which should facilitate an early diagnosis if recognised.^{1–4} Although megaesophagus has been described in dogs with MD, its presence in cats has been inconsistently reported.^{2,10–13} The long-term prognosis for MD is grave. Quality of life may be improved by managing megaesophagus with sildenafil, altering diet

consistency and facilitating gravity-assisted feeding. Prednisolone therapy may assist mobility and reduce muscle pain. The cat described here displayed clinical signs from an early age with classical disease progression. It was humanely euthanased following a uraemic crisis at 2 years of age.

Case description

The kitten was acquired at 6 weeks of age, appearing normal apart from tongue protrusion. Subtle intermittent pelvic limb lameness was noted at 3 months, followed by occasional bunny hopping over subsequent months. At 5 months, pelvic radiographs, feline leukaemia virus and feline immunodeficiency virus serology, and urinalysis were unremarkable. Haematology, biochemistry, *Toxoplasma* IgM and IgG titres, and muscle biopsies were performed by the referring veterinarian, with marked CK elevation and other relevant clinicopathological abnormalities noted in Table 1. Biopsies of the gastrocnemius, semimembranosus and trapezius muscles were submitted to a local laboratory. The histopathological summary diagnosed lymphohistiocytic myositis with myocyte degeneration, necrosis, regeneration and protozoal cysts. Microscopically bradyzoites were identified within a thin-walled cyst multifocally within macrophages. Therapy was initiated as detailed in Table 2. Marked regurgitation, hypersalivation, intermittent coughing and mild oropharyngeal dysphagia were observed over the ensuing weeks. Thoracic radiographs with barium confirmed megaesophagus (Figure 2).

Specialist referral at 8 months identified marked macroglossia (Figure 3a) with reduced lingual dexterity,

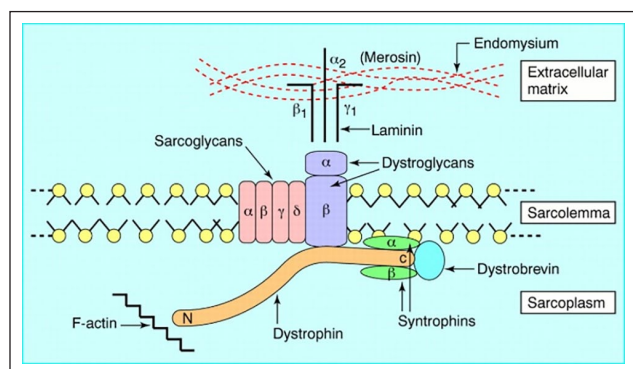


Figure 1 Normal dystrophin–glycoprotein complex and its relationship with the muscle membrane and contractile units⁸

Table 1 Abnormal results* at 5 months, 1 year and 2 years of age from haematology, biochemistry and *Toxoplasma* serology

| Test performed | Results | | | RI |
|----------------------------------------|----------------|----------------|---------------|----------|
| | At 5 months | At 1 year | At 2 years | |
| Creatinine ($\mu\text{mol/l}$) | 40 | 130 | 465 | 80–200 |
| Urea (mmol/l) | 8.9 | 9.6 | 46.2 | 3–10 |
| Phosphate (mmol/l) | 2.6 | 1.76 | 3.1 | 1–2.3 |
| ALT (IU/l) | 633 | 342 | 85 | 19–100 |
| AST (IU/l) | 1953 | 1400 | 450 | 2–62 |
| CK (IU/l) [†] | 218,127 | 156,093 | 38,896 | 64–400 |
| Haematology | | | | |
| WBCs ($\times 10^9/\text{l}$) | 10.2 | N/A | 24.7 | 6–16 |
| Neutrophils ($\times 10^9/\text{l}$) | 5.4 | N/A | 20.7 | 3.8–10.2 |
| HCT (%) | 29 | N/A | 26 | 28–45 |
| <i>Toxoplasma</i> IgG | 512 | N/A | N/A | <1:64 |
| <i>Toxoplasma</i> IgM | <1:16 | N/A | N/A | <1:64 |

*Abnormal results are in bold.

[†]Decline in CK activity was noted with advanced disease due to progressive muscle wastage and fibrosis. Concurrent elevations in ALT are secondary to primary muscle disease, as the magnitude of the increase in ALT is less than that of AST¹⁴

ALT = alanine transferase; AST = aspartate transferase; CK = creatine kinase; HCT = haematocrit; N/A = not available; RI = reference interval; WBC = white blood cell

Table 2 Treatment for presumed *Toxoplasma* myopathy

| Medication administered | Dose | Route | Frequency (h) | Duration (weeks) |
|------------------------------|-----------|-------|---------------|------------------|
| Clindamycin | 8.5 mg/kg | PO | 12 | 4 |
| Toltrazuril | 15 mg/kg | PO | 24 | 4 |
| Trimethoprim sulfonamide | 11 mg/kg | PO | 12 | 4 |
| Gabapentin | 11 mg/kg | PO | 12 | 4 |
| Fortiflora (Purina Pro Plan) | 1 sachet | PO | 24 | 6 |

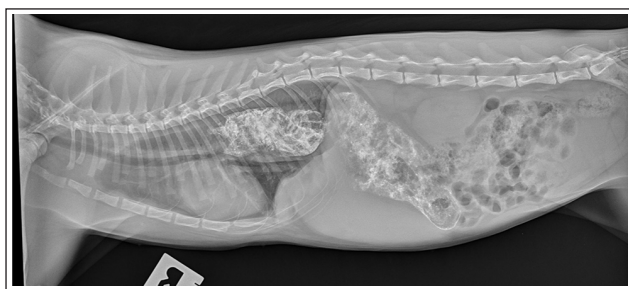


Figure 2 Oesophagram image using barium sulfate (60% w/v) with the cat in right lateral recumbency 30 mins after barium ingestion. The barium was co-administered with dry food. The radiograph also indicates a scalloped appearance of the diaphragm, which is likely due to thickening secondary to muscular dystrophy

hypertrophy of the neck and shoulder muscles (Figure 3b) with mild spinal discomfort on palpation, reduced body condition score (BCS) of 2/5, normothermia and normal cardiac auscultation. Neurological examination revealed mild ambulatory paraparesis, normal to reduced pelvic limb segmental reflexes and a reduced gag reflex. MD was suspected and cardiac troponin I, repeat CK levels and secondary histopathological review were requested. Persistent CK and marked troponin elevation (124 ng/l, reference interval [RI] 0–16) were noted. On review, the histopathologist verbally acknowledged that an error had been made, and no protozoal cysts were seen.

Repeat muscle biopsies for specialised MD immunostains were declined. Omeprazole (1.1 mg/kg PO q24h) and upright feeding were originally initiated to manage the regurgitation and megaesophagus with minimal clinical improvement. Sildenafil liquid (2.8 mg/kg PO q12h) was added with a moderate improvement, showing a 60–70% reduction in the frequency of regurgitation observed.

Gradual-onset diffuse hyperaesthesia, progressive muscle weakness and pain were noted, with peak signs at 11 months of age. Prednisolone was administered (1 mg/kg PO q24h) with moderate improvement in mobility and muscular pain on palpation. Prednisolone was continued at 0.5 mg/kg PO q24h thereafter as

deterioration was noted when the medication was discontinued.

At 16 months of age, a fluoroscopic swallow study using iodinated contrast material (Iohexol; GE Healthcare Australia) revealed a normal pharyngeal and cricopharyngeal phase of swallowing, and the cervical oesophagus had normal tone and appropriate primary and secondary peristaltic activity; however, the thoracic oesophagus was flaccid and distended (megaesophagus) with a lack of peristaltic activity. No conclusive evidence of oesophageal achalasia-like syndrome was detected with liquid contrast material, but the study was incomplete as the authors were unable to administer all consistencies of barium and could give only a limited number of boluses because of the cat's demeanour. Consequently, a partial oesophageal stricture at the thoracic inlet could not be excluded but was deemed less likely. Echocardiography was recommended but declined.

At approximately 2 years of age, after slow progressive deterioration including tongue protrusion (Figure 3c) and generalised weakness, polydipsia, polyuria, abdominal pain and hyporexia developed. A physical examination confirmed abdominal pain, moderate pyrexia (39.9°C), moderate dehydration, marked muscle hypertrophy, reduced BCS of 1.5/5 and lingual calcific deposits (Figure 4). Urinalysis identified isosthenuria with a positive urine culture for a multidrug-resistant *Pseudomonas aeruginosa*. Marked azotaemia and hyperphosphataemia were noted (see Table 1). Abdominal ultrasound showed bilaterally reduced renal corticomedullary distinction, with increased echogenicity of the deeper cortex and mild renal pylectasia (Figure 5). The gastrointestinal tract was unremarkable and no diaphragmatic imaging was performed. Findings were suggestive of acute-on-chronic kidney disease, with presumed pyelonephritis. Despite 5 days of hospitalisation with intravenous fluid therapy, antimicrobials and supportive care, no significant improvement was observed.

The progression of MD signs and renal disease led to euthanasia. Unfixed and fixed muscle samples collected post mortem from the biceps femoris, epaxial, triceps, tongue, neck and heart muscles were

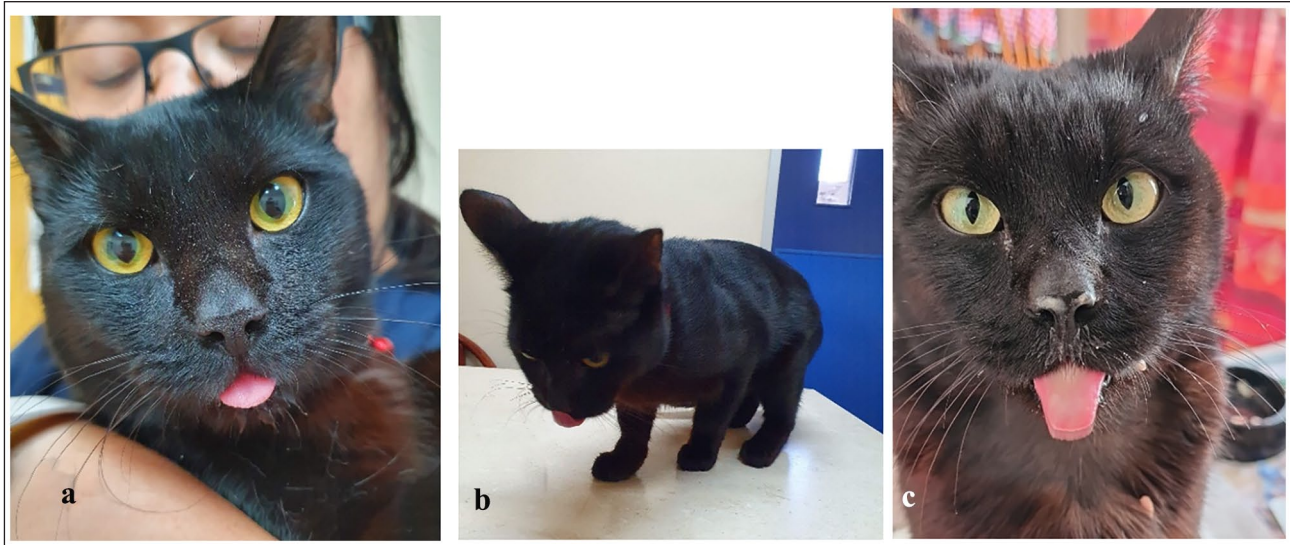


Figure 3 (a) Tongue size at 8 months old; (b) shoulder hypertrophy and (c) tongue size at 24 months old



Figure 4 Lingual calcium deposits, confirmed with alizarin reaction on histopathology. Calcinosis circumscripta has been a previously noted feature of cats with dystrophin-deficient MD and can be another clinical feature to prompt investigation for potential MD.¹² MD = muscular dystrophy

transported chilled by courier to the Comparative Neuromuscular Laboratory, San Diego, USA, for evaluation by standard histological and histochemical stains and reactions. A dystrophic phenotype was present in all muscles evaluated and included multifocal clusters of necrotic (degenerating) fibres with phagocytosis, clusters of regenerating fibres and numerous calcific deposits (Figure 6). Immunofluorescence staining of muscle cryosections using monoclonal antibodies including those against the rod and carboxy terminus of dystrophin, laminin $\alpha 2$, utrophin and spectrin, and against α -, beta (β)- and gamma (γ)-sarcoglycans confirmed dystrophin-deficient MD (Figure 7) with secondary reductions in dystrophin-associated proteins



Figure 5 Renal ultrasound at 2 years of age: reduced corticomedullar distinction, increased cortical echogenicity

and glycoproteins. Spectrin staining as a control indicated good tissue quality.

Discussion

The clinical presentation at 8 months showed several distinct phenotypic features strongly associated with MD in cats, including marked lingual and shoulder muscle hypertrophy. These findings combined with marked hyperCKaemia and troponin I elevation may be considered pathognomonic for this condition.

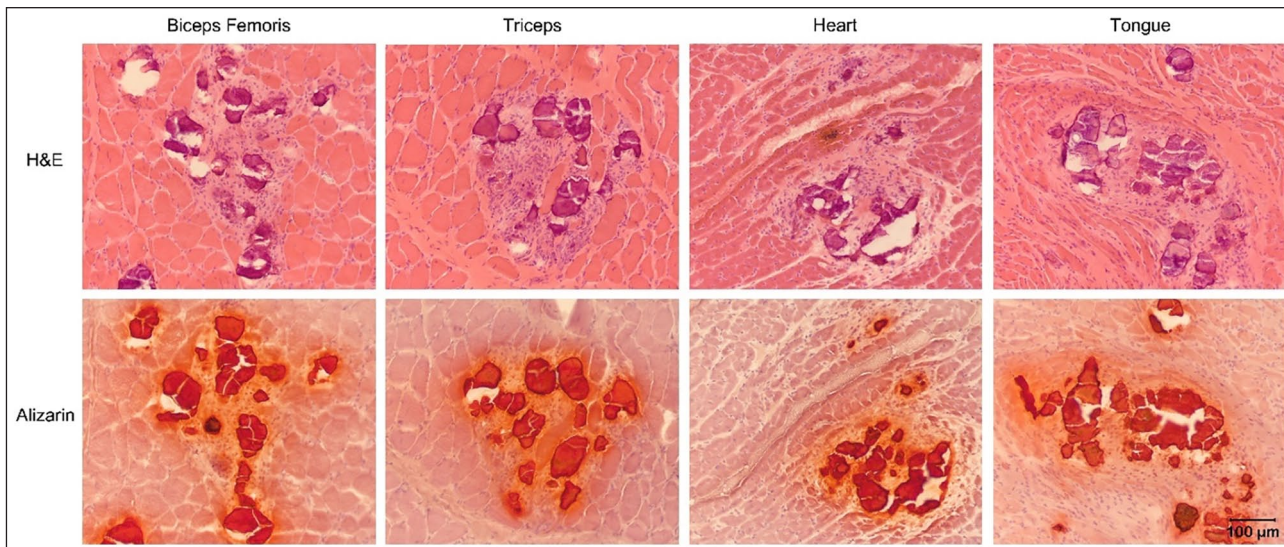


Figure 6 Haematoxylin & eosin (H&E) and alizarin staining for calcific deposits

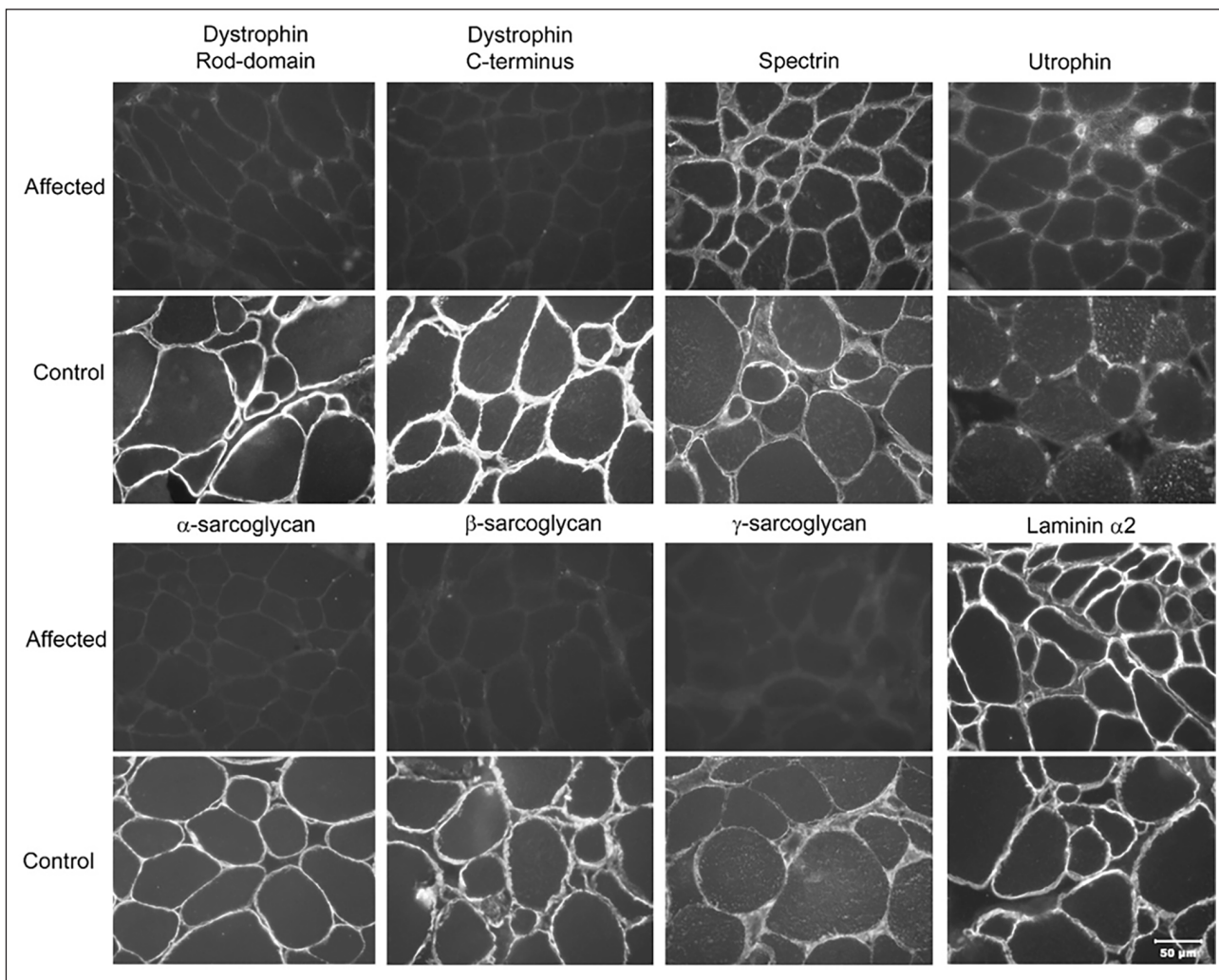


Figure 7 Immunofluorescence staining of muscle cryosections using monoclonal antibodies against dystrophin-associated proteins

Megaoesophagus has been described with canine MD, being noted in 34.2% of thoracic radiographs of Golden Retrievers with MD in one study.¹⁵ Regrettably, in the present case, sections of the oesophagus and diaphragm were not collected at the post-mortem examination to determine whether the MD was associated with myofibre degeneration of the thoracic and abdominal oesophageal muscles or diaphragm, as noted in a Spanish Water Spaniel.¹⁶ The use of clindamycin to treat the misdiagnosed *Toxoplasma* infection was considered as a possible cause of oesophageal injury; however, a classical stricture was not observed on swallow fluoroscopy and oesophagoscopy was declined.

Disease progression was observed with the development of dysphagia secondary to progressive lingual hypertrophy and megaoesophagus, leading to altered food and water intake and loss of body condition. Upright feeding was essential to reduce the risk of regurgitation and potential for aspiration pneumonia. Given the inconsistent finding of megaoesophagus in feline MD, a standing fluoroscopic swallow study was performed confirming megaoesophagus of the intrathoracic segment, with preserved cervical function.

The phosphodiesterase inhibitor sildenafil has been used in people and dogs for the management of megaoesophagus with oesophageal achalasia.^{17,18} It has variable success, with some reports documenting benefit in the management of canine congenital idiopathic megaoesophagus¹⁹ and others showing no difference in oesophageal clearance time or quality-of-life scores between sildenafil and placebo.¹⁷ Clinical improvement was noted in the present cat after starting sildenafil, therefore it was presumptively beneficial. Serial radiographs may have been considered to monitor oesophageal diameter. The use of sildenafil, with concurrent upright feeding and use of omeprazole for presumed oesophagitis, may be considered in other cases of feline MD with megaoesophagus.

Uraemia, progressive regurgitation and weakness resulted in euthanasia. In human patients with MD, renal disease occurs secondarily to advanced MD due to insufficient water intake, dehydration and hyperosmolar syndrome, leading to acute renal failure.²⁰ As survival times increase, the identification of chronic kidney disease is increased in people.²⁰ The renal pathology aetiology was not elucidated in this patient; however, pyelonephritis was suspected, possibly exacerbated by prolonged prednisolone use.

Gashen et al²¹ showed male cats with MD had a higher predisposition to developing clinical cardiac disease. Although severe elevation in cardiac troponin I and abnormal cardiac histopathology were identified, testing for N-terminal pro-B-type natriuretic peptide (Cardiopet proBNP; IDEXX) was normal and clinical cardiac disease was not observed. However, all cats with suspected MD should be evaluated by echocardiogram,

including siblings and female cats of the same family line, and not used for breeding.²¹

The original misdiagnosis occurred as suspected *Toxoplasma* bradyzoites were identified on routine paraffin histopathology. It is surmised that the pathologist may have received a truncated history, indicating the patient was *Toxoplasma* positive, without extensive details. This highlights the importance of a solid understanding of *Toxoplasma* serology interpretation, the need for further enquiry if findings do not correlate with the clinical picture and the fundamental use of immunofluorescence staining to obtain a definitive diagnosis of MD. Toxoplasmosis was discounted through failed clinical response, discussion with the pathologist and re-review, many months later. It is assumed that the positive IgG titre was due to previous exposure rather than current infection. A repeat and rising titre was not demonstrated.

Extrapolation from human medicine implies treatment with prednisolone could be beneficial in the early stages of MD.^{22,23} In people, prednisone therapy is recommended to delay disease progression via slowed muscle loss and extended ambulation.²²⁻²⁵ Liu et al²⁶ evaluated the use of prednisolone in Golden Retrievers that showed clinical improvement but histological decline. A recent review evaluating the effects of daily glucocorticoid therapy in boys with Duchenne MD confirmed a beneficial response.²⁴

Conclusions

This cat was diagnosed with dystrophin-deficient MD with a classical clinical disease presentation and progression including muscular and glossal hypertrophy, cardiomyopathy and dysphagia. Megaoesophagus and subsequent regurgitation were successfully managed with sildenafil, soft food and vertical feeding. Prednisolone therapy assisted ambulation with suspected reduction in muscle loss and discomfort but could have been initiated earlier with a correct diagnosis. This case highlights the importance of performing muscle biopsies in animals with hyperCKaemia and the diagnostic utility of immunohistochemical testing for the detection of many proteins that result in MD, including, but not limited to, dystrophin, sarcoglycans, laminin $\alpha 2$, dysferlin, α - and β -dystroglycans, utrophin and spectrin. In addition, this case highlights the need to recognise MD as an important consideration for a cat showing marked macroglossia, lingual calcium deposits, muscular hypertrophy and marked persistent hyperCKaemia. Screening for megaoesophagus should be included for targeted therapy to optimise the patient's quality of life.

Supplementary material The following files are available as supplementary material:

Muscle profile and peripheral nerve profile.

Dystrophy panel report.

Histopathology report.

Conflict of interest The authors declared no potential conflicts of interest with respect to the research, authorship, and/or publication of this article.

Funding The authors received no financial support for the research, authorship, and/or publication of this article.

Ethical approval The work described in this manuscript involved the use of non-experimental (owned or unowned) animals. Established internationally recognised high standards ('best practice') of veterinary clinical care for the individual patient were always followed and/or this work involved the use of cadavers. Ethical approval from a committee was therefore not specifically required for publication in *JFMS Open Reports*. Although not required, where ethical approval was still obtained, it is stated in the manuscript.

Informed consent Informed consent (verbal or written) was obtained from the owner or legal custodian of all animal(s) described in this work (experimental or non-experimental animals, including cadavers) for all procedure(s) undertaken (prospective or retrospective studies). For any animals or people individually identifiable within this publication, informed consent (verbal or written) for their use in the publication was obtained from the people involved.

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References

- Beckers E, Cornelis I, Bhatti SFM, et al. **A nonsense variant in the DMD gene causes X-linked muscular dystrophy in the Maine Coon cat.** *Animals (Basel)* 2022; 12. DOI: 10.3390/ani12212928.
- Gaschen FP, Hoffman EP, Gorospe JR, et al. **Dystrophin deficiency causes lethal muscle hypertrophy in cats.** *J Neurol Sci* 1992; 110: 149–159.
- Schatzberg SJ and Shelton GD. **Newly identified neuromuscular disorders.** *Vet Clin North Am Small Anim Pract* 2004; 34: 1497–1524.
- Shelton GD and Engvall E. **Muscular dystrophies and other inherited myopathies.** *Vet Clin North Am Small Anim Pract* 2002; 32: 103–124.
- Poncelet L, Résibois A, Engvall E, et al. **Laminin alpha2 deficiency-associated muscular dystrophy in a Maine Coon cat.** *J Small Anim Pract* 2003; 44: 550–552.
- Martin PT, Shelton GD, Dickinson PJ, et al. **Muscular dystrophy associated with alpha-dystroglycan deficiency in Sphynx and Devon Rex cats.** *Neuromuscul Disord* 2008; 18: 942–952.
- Salvadori C, Vattemi G, Lombardo R, et al. **Muscular dystrophy with reduced beta-sarcoglycan in a cat.** *J Comp Pathol* 2009; 140: 278–282.
- Emery AEH. **The muscular dystrophies.** *BMJ* 1998; 317: 991. DOI: 10.1136/bmj.317.7164.991.
- Hall JE and Hall ME. *Guyton and Hall textbook of medical physiology*. 14th ed. Philadelphia: Elsevier, 2021, pp 78–92.
- Carpenter JL, Hoffman EP, Romanul FCA, et al. **Feline muscular dystrophy with dystrophin deficiency.** *Am J Pathol* 1989; 135: 909–919.
- Berry CR, Gaschem FP and Ackerman N. **Radiographic and ultrasonographic features of hypertrophic feline muscular dystrophy in two cats.** *Vet Radiol Ultrasound* 1992; 33: 357–364.
- Eiras-Diaz A, Prisco F, Paciello O, et al. **Unusual clinical presentation of dystrophin-deficient feline muscular dystrophy in the UK.** *Vet Rec Case Rep* 2020; 8. DOI: 10.1136/vetreccr-2019-000983.
- Gambino AN, Mounser PJ, Shelton GD, et al. **Emergent presentation of a cat with dystrophin-deficient muscular dystrophy.** *J Am Anim Hosp Assoc* 2014; 50: 130–135.
- Valentine BA, Blue JT, Shelley SM, et al. **Increased serum alanine aminotransferase activity associated with muscle necrosis in the dog.** *J Vet Intern Med* 1990; 4: 140–143.
- Bedu AS, Labruyere JJ, Thibaud JL, et al. **Age-related thoracic radiographic changes in Golden and Labrador Retriever muscular dystrophy.** *Vet Radiol Ultrasound* 2012; 53: 492–500.
- McAtee BB, Heseltine JC, Guo LT, et al. **Dysphagia and esophageal dysfunction due to dystrophin deficient muscular dystrophy in a male Spanish water spaniel.** *Vet Q* 2018; 38: 28–32.
- Mehain SO, Haines JM and Guess SC. **A randomized crossover study of compounded liquid sildenafil for treatment of generalized megaesophagus in dogs.** *Am J Vet Res* 2022; 83: 317–323.
- Bortolotti M, Mari C, Lopilato C, et al. **Effects of sildenafil on esophageal motility of patients with idiopathic achalasia.** *Gastroenterology* 2000; 118: 253–257.
- Quintavalla F, Menozzi A, Pozzoli C, et al. **Sildenafil improves clinical signs and radiographic features in dogs with congenital idiopathic megaesophagus: a randomised controlled trial.** *Vet Rec* 2017; 180: 404. DOI: 10.1136/vr.103832.
- Motoki T, Shimizu-Motohashi Y, Komani H, et al. **Renal dysfunction can occur in advanced-stage Duchenne muscular dystrophy.** *Muscle Nerve* 2020; 61: 192–197.
- Gashen L, Lang J, Lin S, et al. **Cardiomyopathy in dystrophin-deficient hypertrophic feline muscular dystrophy.** *J Vet Intern Med* 1999; 13: 346–356.
- Matthews E, Brassington R, Kuntzer T, et al. **Corticosteroids for the treatment of Duchenne muscular dystrophy.** *Cochrane Database Syst Rev* 2016; 2016. DOI: 10.1002/14651858.CD003725.pub4.
- Angelini C. **The role of corticosteroids in muscular dystrophy: a critical appraisal.** *Muscle Nerve* 2007; 36: 424–435.
- Kochar GS, Sondhi V, Kabra SK, et al. **Intermittent versus daily regimen of prednisolone in ambulatory boys with Duchenne muscular dystrophy: a randomized, open-label trial.** *Muscle Nerve* 2022; 65: 60–66.
- Yilmaz O, Karaduman A and Topaloğlu H. **Prednisolone therapy in Duchenne muscular dystrophy prolongs ambulation and prevents scoliosis.** *Europ J Neurol* 2004; 11: 541–544.
- Liu JMK, Okamura CS, Bogan DJ, et al. **Effects of prednisolone in canine muscular dystrophy.** *Muscle Nerve* 2004; 30: 767–773.